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

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of avian polyclonal IgY antibody against *Pseudomonas aeruginosa* for the treatment of cystic fibrosis

On 23 September 2008, orphan designation (EU/3/08/564) was granted by the European Commission to Immunsystem I.M.S. AB, Sweden, for avian polyclonal IgY antibody against *Pseudomonas aeruginosa* for the treatment of cystic fibrosis.

What is 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.
Cystic fibrosis is a long lasting and life-threatening disease.

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 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 65,000 people.

What treatments are available?
At the time of submission of the application for orphan drug designation, lung infection and inflammation in cystic fibrosis were mainly treated with physiotherapy and antibiotics. Other medicines used to treat the lung disease included bronchodilators (medicines that help to open up the airways in the lungs) and mucolytics (medicines that help dissolve the mucus in the lungs). In addition, patients are often given other types of medicine such as pancreatic enzymes (substances that help to digest and absorb food) and food supplements. They are also advised to exercise and to undergo physiotherapy.
Satisfactory argumentation has been submitted by the sponsor to justify the assumption that avian polyclonal IgY antibody against *Pseudomonas aeruginosa* might be of potential significant benefit for the treatment of cystic fibrosis because it has a new mechanism of action. This assumption will have to

[*]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 (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

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be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?
This medicine is a solution that contains antibodies targeting *Pseudomonas aeruginosa*, the bacterium that causes most lung disease in patients with cystic fibrosis. The antibodies are made in the eggs of hens that have been vaccinated against *P. aeruginosa*. After the patient gargles with the solution, the antibodies are expected to coat the bacteria, preventing them from sticking to the cells lining the mouth and the throat. This is expected to reduce the transfer of the bacteria to the lungs and prevent lung infection.

What is the stage of development of this medicine?
The effects of avian polyclonal IgY antibody against *P. aeruginosa* have been evaluated in experimental models.

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

The medicinal product was not authorised anywhere worldwide for condition or designated as an 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 a positive opinion on 9 July 2008 recommending the granting 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.

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

Language	Active Ingredient	Indication
English	Avian polyclonal IgY antibody against *Pseudomonas aeruginosa*	Treatment of cystic fibrosis
Bulgarian	Птиче поликлонално IgY антитяло срещу *Pseudomonas aeruginosa*	Лечение на кистозна фиброза
Czech	Ptačí polyklonální IgY protilátka proti Pseudomonase aeruginosa	Léčba cystické fibrózy
Danish	Aviært polyklonalt IgY antistof mod *Pseudomonas aeruginosa*	Behandling af cystisk fibrose
Dutch	Aviair polyklonaal IgY antilichaam gericht tegen *Pseudomonas aeruginosa*	Behandeling van cystische fibrose
Estonian	*Pseudomonas aeruginosa* vastane linnu polüklonaalne IgY antikeha	Tsüstilise fibroosi ravi
Finnish	Linnun polyklonaalinen IgY vasta-aine *Pseudomonas aeruginosaa* vastaan	Kystisen fibroosin hoito
French	Anticorps polyclonal IgY aviaire contre le *Pseudomonas Aeruginosa*	Traitement de la mucoviscidose
German	Avianer polyklonaler IgY Antikörper gegen Pseudomonas Aeruginosa	Behandlung zystischer Fibrose
Greek	Πολυκλονικό αντίσωμα IgY πτηνών ενάντια στην πσευδομονάδα του κυανού πύου	Θεραπεία της κυστικής ίνωσης
Hungarian	Szárnyasból származó, *Pseudomonas aeruginosa*-ellenes poliklonális IgY antitest	Cisztikus fibrózis kezelése
Italian	Anticorpo aviario policlonale IgY anti-*Pseudomonas aeruginosa*	Trattamento della fibrosi cistica
Latvian	Putnu poliklonālās IgY antivielas pret *Pseudomonas aeruginosa*	Cistiskās fibrozes ārstēšana
Lithuanian	Paukščių polikloninis IgY antikūnas prieš *Pseudomonas aeruginosa*	Cistinės fibrozės gydymas
Maltese	Antikorp IgY polikonali ta' l-għasafar kontra l-*Pseudomonas aeruginosa*	Kura tal-fibrożi ċistiku
Polish	Ptasie poliklonalne IgY przeciwciała przeciwko pałeczce ropy błękitnej	Leczenie zwłóknienia torbielowatego
Portuguese	Anticorpo policolonal IgY de origem aviária anti Pseudomonas aeruginosa	Tratamento da fibrose quística
Romanian	Anticorp policlonal aviar IgY anti *Pseudomonas aeruginosa*	Tratamentul fibrozei chistice
Slovak	Vtáčia polyklonálna protilátka IgY proti *Pseudomonas aeruginosa*	Terapia cystickej fibrózy
Slovenian	Aviarno poliklonalno IgY protitelo proti *Pseudomonas aeruginosa*	Zdravljenje cistične fibroze
Spanish	Anticuerpo policlonal IgY de origen aviario contra *Pseudomonas aeruginosa*	Tratamiento de la fibrosis quística
Swedish	Polyklonal fågel IgY antikropp riktat mot *Pseudomonas aeruginosa*	Behandling av cystisk fibros
Norwegian	Aviært polyklonalt IgY antistoff mot *Pseudomonas aeruginosa*	Behandling av cystisk fibrose
Icelandic	Fjölklóna fugla IgY mótefni gegn *Pseudomonas aeruginosa*	Meðferð við slímseigjusjúkdómi