



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human monoclonal antibody against *Pseudomonas aeruginosa* IATS-O1 for the treatment of pneumonia caused by serotype O1 *Pseudomonas aeruginosa*

On 28 January 2010, orphan designation (EU/3/09/705) was granted by the European Commission to Investia Limited, United Kingdom, for human monoclonal antibody against *Pseudomonas aeruginosa* IATS-O1 for the treatment of pneumonia caused by serotype O1 *Pseudomonas aeruginosa*.

What is pneumonia caused by serotype O1 *Pseudomonas aeruginosa*?

Pneumonia (infection of the lungs) can be caused by the bacterium *Pseudomonas aeruginosa* (*P. aeruginosa*). The bacterium is widespread in nature and is able to cause infections in people who are seriously ill, such as patients who are in intensive care (especially those who are unable to breathe by themselves) or people whose immune system (the body's natural defences) is weakened. These patients often catch the disease while they are in hospital. The infection is difficult to cure because *P. aeruginosa* is resistant to many antibiotics.

Serotype O1 *P. aeruginosa* is a sub-group of bacteria that carry a specific type of lipopolysaccharide (a type of sugar) on their surface.

Pneumonia caused by serotype O1 *P. aeruginosa* is a long-term debilitating disease because it lengthens the time that patients spend in hospital. The disease may also be life threatening because the patients affected are often already very weak and have an increased mortality.

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

At the time of designation, pneumonia caused by serotype O1 *P. aeruginosa* affected approximately 1.8 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 91,000 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).

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).



What treatments are available?

At the time of submission of the application for orphan drug designation, several antibiotics were authorised in the EU to treat this condition. Patients with pneumonia that was suspected to be caused by *P. aeruginosa* were given treatment consisting of at least two antibiotics known to be effective against *P. aeruginosa*. These included carbapenems, cephalosporins, piperacillin with tazobactam and fluoroquinolones.

The sponsor has provided sufficient information to show that human monoclonal antibody against *P. aeruginosa* IATS-O1 might be of significant benefit for patients with pneumonia caused by serotype O1 *P. aeruginosa* because it works in a different way to existing treatments, and early studies in experimental models indicate that it might be used together with antibiotics to improve the treatment of patients with this condition. 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?

Human monoclonal antibody against *P. aeruginosa* IATS-O1 is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to the lipopolysaccharide that is found on the surface of serotype O1 *P. aeruginosa*. Once the product is attached to the bacterium, it attracts phagocytes (specialised 'scavenger' cells of the immune system) that attack and dispose of the bacterium.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of human monoclonal antibody against *P. aeruginosa* IATS-O1 in experimental models was ongoing.

At the time of submission, no clinical trials with the designated product in patients with pneumonia caused by serotype O1 *P. aeruginosa* had been started.

At the time of submission, human monoclonal antibody against *P. aeruginosa* IATS-O1 was not authorised anywhere in the EU for pneumonia caused by serotype O1 *P. aeruginosa* or designated as an 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 5 November 2009 recommending the granting of this 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;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the Community) 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

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Patient associations' contact points

None available.

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

Language	Active ingredient	Indication
English	Human monoclonal antibody against <i>Pseudomonas aeruginosa</i> IATS-O1	Treatment of pneumonia caused by serotype O1 <i>Pseudomonas aeruginosa</i>
Bulgarian	Човешко моноклонално антитяло срещу <i>Pseudomonas aeruginosa</i> , серотип IATS-O1	Лечение на пневмония причинена от <i>Pseudomonas aeruginosa</i> , серотип O1
Czech	Lidská monoklonální protilátka proti <i>Pseudomonas aeruginosa</i> sérotypu IATS-O1	Léčba pneumonie způsobené <i>Pseudomonadou aeruginosa</i> sérotypu O1
Danish	Humant monoklonalt antistof mod <i>Pseudomonas aeruginosa</i> serotype IATS-O1	Behandling af pneumoni forårsaget af serotype O1 <i>Pseudomonas aeruginosa</i>
Dutch	Humaan monoklonaal antilichaam tegen <i>Pseudomonas aeruginosa</i> serotype IATS-O1	Behandeling van pneumonia veroorzaakt door serotype O1 <i>Pseudomonas aeruginosa</i>
Estonian	Inimese monoklonaalne <i>Pseudomonas aeruginosa</i> serotüübi IATS-O1 vastane antikeha	Serotüüp O1 <i>Pseudomonas aeruginosa</i> põhjustatud pneumoonia ravi
Finnish	Ihmisen monoklonaalinen vasta-aine <i>Pseudomonas aeruginosa</i> serotyyppiä IATS-O1 vastaan	<i>Pseudomonas aeruginosa</i> serotyypin O1:n aiheuttaman pneumonian hoito
French	Anticorps monoclonal humain contre le <i>Pseudomonas aeruginosa</i> de sérotype IATS-O1	Traitement de la pneumonie causée par le <i>Pseudomonas aeruginosa</i> de sérotype O1
German	Humaner monoklonaler Antikörper gegen <i>Pseudomonas aeruginosa</i> Serotyp IATS-O1	Behandlung von durch Serotyp O1 <i>Pseudomonas aeruginosa</i> verursachter Pneumonie
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα κατά του ορότυπου IATS-O1 της ψευδομονάδας <i>aeruginosa</i>	Θεραπευτική αγωγή πνευμονίας που προκλήθηκε από ορότυπο O1 της ψευδομονάδας <i>aeruginosa</i>
Hungarian	<i>Pseudomonas aeruginosa</i> IATS-O1 szerotípus elleni humán monoklonális antitest	<i>Pseudomonas aeruginosa</i> O1 szerotípus által okozott pneumónia kezelés
Italian	Anticorpi monoclonali umani contro il serotipo <i>Pseudomonas aeruginosa</i> IATS-O1	Terapia della polmonite provocata da <i>Pseudomonas aeruginosa</i> serotipo O1
Latvian	Cilvēka monoklonālās antivielas pret <i>Pseudomonas aeruginosa</i> serotipu IATS-O1	<i>Pseudomonas aeruginosa</i> serotipa O1 izraisītas pneimonijas ārstēšana
Lithuanian	Žmogaus monokloninis antikūnas prieš <i>Pseudomonas aeruginosa</i> IATS-O1	Pneumonijos, sukeltos <i>Pseudomonas aeruginosa</i> O1 serotipo, gydymas
Maltese	Antikorp monoklonali uman kontra s-serotip IATS-O1 ta' <i>Pseudomonas aeruginosa</i>	Kura tal-pnewmonja ikkawżata mis-serotip O1 ta' <i>Pseudomonas aeruginosa</i>
Polish	Ludzkie przeciwciało monoklonalne przeciw <i>Pseudomonas aeruginosa</i> serotyp IATS-O1	Leczenie zapalenia płuc wywołanego <i>Pseudomonas aeruginosa</i> (serotyp O1)
Portuguese	Anticorpo monoclonal humano anti <i>Pseudomonas aeruginosa</i> serotipo IATS-O1	Tratamento de Pneumonia causada pelo serotipo O1 da <i>Pseudomonas aeruginosa</i>
Romanian	Anticorp monoclonal uman anti – <i>Pseudomonas aeruginosa</i> serotipul IATS-O1	Tratamentul pneumoniei produsă de <i>Pseudomons aeruginosa</i> serotipul O1

Language	Active ingredient	Indication
Slovak	Ľudská monoklonálna protilátka proti <i>Pseudomonas aeruginosa</i> sérotypu IATS-O1	Liečba pneumónie spôsobenej <i>Pseudomonas aeruginosa</i> sérotypu O1
Slovenian	Humanizirano monoklonsko protitelo, usmerjeno proti bakteriji <i>Pseudomonas aeruginosa</i> serotipa IATS-O1	Zdravljenje okužbe ki jo povzroča serotip O1 bakterije <i>Pseudomonas aeruginosa</i>
Spanish	Anticuerpo monoclonal humano contra <i>Pseudomonas aeruginosa</i> serotipo IATS-O1	Tratamiento de la neumonía causada por <i>Pseudomonas aeruginosa</i> serotipo O1
Swedish	Mänsklig monoklonal antikropp mot <i>Pseudomonas aeruginosa</i> serotyp IATS-O1	Behandling av pneumoni orsakad av serotyp O1 <i>Pseudomonas aeruginosa</i>
Norwegian	Humant monoklonalt antistoff mot <i>Pseudomonas aeruginosa</i> serotype IATS-O1	Behandling av pneumoni forårsaket av serotype O1 <i>Pseudomonas aeruginosa</i>
Icelandic	Manna einstofna mótefni gegn <i>Pseudomonas aeruginosa</i> af sermisgerð IATS-O1	Meðferð við <i>Pseudomonas aeruginosa</i> sermisgerð O1 lungnabólgu