



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 Fas ligand for the treatment of pemphigus

On 9 February 2012, orphan designation (EU/3/12/956) was granted by the European Commission to PinCell s.r.l., Italy, for human monoclonal antibody against Fas ligand for the treatment of pemphigus.

What is pemphigus?

Pemphigus is a group of autoimmune diseases characterised by widespread blistering and erosion of the skin and mucous membranes (the linings of internal organs). 'Autoimmune' means that the disease is caused by the immune system attacking the body's own cells.

In pemphigus, the autoimmune response results in damage to desmoglein, a protein which acts as a 'glue' attaching together the cells of the epidermis (the outer layer of the skin). This causes skin cells to separate from each other, causing blisters that turn into sores. In some cases these blisters can cover large areas of the skin.

Pemphigus is a long-term debilitating and life-threatening disease because of the long-term blistering which can lead to dehydration and infection.

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

At the time of designation, pemphigus affected approximately 2.4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 122,000 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, prednisolone (a steroid) and azathioprine (a medicine that reduces the activity of the immune system) were authorised in the EU to treat pemphigus.

*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 506,300,000 (Eurostat 2011).



The sponsor has provided sufficient information to show that human monoclonal antibody against Fas ligand might be of significant benefit for patients with pemphigus because early studies show that it may be able to help prevent the blistering and skin damage seen in 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a naturally occurring protein called 'human Fas ligand'. Skin cells release this protein when they are 'attacked' by a defective immune system. People with pemphigus have high levels of Fas ligand, which is believed to result in damage to desmoglein. By attaching to Fas ligand, this medicine is expected to prevent its action, thereby reducing the blistering seen in people with this condition.

What is the stage of development of this medicine?

The effects of human monoclonal antibody against Fas ligand have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with this medicine in patients with pemphigus had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for pemphigus 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 7 December 2011 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 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:

PinCell s.r.l
via del Pozzo 71
41124 Modena (MO)
Italy
Telephone: +39 059 4222931
Telefax: +39 059 4224271
E-mail: info@pincell.it

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	Human monoclonal antibody against Fas ligand	Treatment of pemphigus
Bulgarian	Човешко моноклонално антитяло срещу Fas-лиганда	Лечение на пемфигус
Czech	Humánní monoklonální protilátka proti Fas ligandu	Léčba pemphigu
Danish	Humant monoklonalt antistof mod Fas-ligand	Behandling af pemphigus
Dutch	Humaan monoklonaal antilichaam tegen Fas-ligand	Behandeling van pemphigus
Estonian	Fas-ligandi vastane inimese monoklonaalne antikeha	Pemfiguse ravi
Finnish	Ihmisen Fas-ligandin monoklonaalinen vasta-aine	Pemfiguksen hoito
French	Anticorps monoclonal humain dirigé contre le ligand de Fas (Fas-L)	Traitement du pemphigus
German	Humaner monoklonaler Antikörper gegen Fas-Ligand	Behandlung des Pemphigus
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα έναντι του προσδέματος Fas	Θεραπεία της πέμφιγας
Hungarian	Fas-ligand elleni humán monoklonális antitest	Pemphigus kezelése
Italian	Anticorpo monoclonale umano anti-Fas-ligando	Trattamento del pemfigo
Latvian	Cilvēka monoklonāla antivielā pret Fas-ligandu	Pemfigusa (pemphigus) ārstēšana
Lithuanian	Žmogaus monokloninis antikūnis prieš Fas ligandą	Pūslinės (pemphigus) gydymas
Maltese	Antikorp monoklonali uman kontra Fas Ligand	Kura ta' Pemfigus
Polish	Ludzkie przeciwciało monoklonalne przeciwko ligandowi Fas	Leczenie pęcherzycy
Portuguese	Anticorpo monoclonal humano anti-Fas Ligante	Tratamento do pênfigo
Romanian	Anticorp monoclonal uman împotriva ligandului Fas	Tratamentul pemfigusului
Slovak	Ľudská monoklonálna protilátka proti Fas ligandu	Liečba pemfigu
Slovenian	Človeško monoklonalno protitelo proti FAS ligand	Zdravljenje pemfigusa
Spanish	Anticuerpo monoclonal humano dirigido contra el ligando de Fas	Tratamiento del pénfigo
Swedish	Human monoklonal antikropp mot FAS-ligand	Behandling av pemfigus
Norwegian	Humant monoklonalt antistoff mot Fas ligand	Behandling av pemfigus
Icelandic	Einstofna manna mótefni gegn Fas bindli	Meðferð við blöðrusótt

¹ At the time of designation