



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

22 January 2018
EMA/COMP/737372/2017

Public summary of opinion on orphan designation

Agammaglobulinaemia tyrosine kinase for the treatment of pemphigus

On 12 December 2017, orphan designation (EU/3/17/1951) was granted by the European Commission to Clinical Network Services (UK) Ltd, United Kingdom, for agammaglobulinaemia tyrosine kinase (also known as PRN1008) 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 (the body's natural defences) attacking the body's own cells.

In pemphigus, the immune system produces antibodies that attack desmoglein, a protein which acts as a 'glue' attaching the cells of the epidermis (the outer layer of the skin). As a result skin cells 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 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 72,000 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 corticosteroid) 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 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pemphigus because early studies show that it may reduce the need for corticosteroids in some patients. 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 reduces the activity of an enzyme known as Bruton tyrosine kinase (BTK). This enzyme activates certain immune cells including those involved in pemphigus. By reducing BTK's activity, this medicine is expected to reduce the autoimmune response in patients with pemphigus, thus improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for pemphigus. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 31 October 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	Agammaglobulinaemia tyrosine kinase	Treatment of pemphigus
Bulgarian	Тирозинкиназна агамаглобулинемия	Лечение на пемфигус
Croatian	Agamaglobulinemija tirozin kinaza	Liječenje pemfigusa
Czech	Agamaglobulinemie tyrosin kinázy	Léčba pemfigu
Danish	Agammaglobulinæmityrosinkinase	Behandling af pemphigus
Dutch	Agammaglobulinaemia tyrosinekinase	Behandeling van pemphigus
Estonian	Agammaglobulineemia türosiinkinaas	Pemfiguse ravi
Finnish	Tyrosiinikinaasi agammaglobulinemia	Pemfiguksen hoito
French	tyrosine kinase agammaglobulinemia	Traitement du pemphigus
German	Agammaglobulinämie Tyrosin Kinase	Behandlung des Pemphigus
Greek	Τυροσινική κινάση αγαμμασφαιριναιμίας	Θεραπεία της πέμφιγας
Hungarian	Agammaglobulinémia tirozin kináz	Pemphigus kezelése
Italian	Tirosin chinasi agammaglobulinemia	Trattamento del pemfigo
Latvian	Agammaglobulinēmijas tirozīna kināze	Pemfigusa (pemphigus) ārstēšana
Lithuanian	Agamaglobulinemijos tirozinkinazė	Pūslinės (pemphigus) gydymas
Maltese	Kinażi tat-tirożina agammaglobulinemja	Kura ta' Pemfigus
Polish	Kinaza tyrozynowa agammaglobulinemii	Leczenie pęcherzycy
Portuguese	Agamaglobulinemia tirosina quinase	Tratamento do pênfigo
Romanian	Agammaglobulinemia tirozin kinază	Tratamentul pemfigusului
Slovak	Agammaglobulinémia tyrozín kináza	Liečba pemfigu
Slovenian	Agamaglobulinska tirozin kinaza	Zdravljenje pemfigusa
Spanish	Tirosina quinasa de agammaglobulinemia	Tratamiento del pénfigo
Swedish	Agammaglobulinämi tyrosin kinas	Behandling av pemfigus
Norwegian	Agammaglobulinemi tyrosin kinase	Behandling av pemfigus
Icelandic	Agammaglóbúlíndreyra týrósin kínasi	Meðferð við blöðrusótt

¹ At the time of designation