



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Bertilimumab for the treatment of bullous pemphigoid

On 24 August 2018, orphan designation (EU/3/18/2062) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for bertilimumab for the treatment of bullous pemphigoid.

What is bullous pemphigoid?

Bullous pemphigoid is an autoimmune skin disease mainly seen in elderly people that is characterised by the development of widespread, itchy blisters affecting the skin, and sometimes the inside of the mouth. 'Autoimmune' means that the disease is caused by the immune system attacking the body's own cells.

In bullous pemphigoid, the immune system produces antibodies that attack proteins in the skin that act as a 'glue' attaching the cells of the epidermis (the outer layer of the skin) to the dermis (lower layer of the skin). As a result skin layers separate from each other and inflammation develops, causing blisters that can turn into sores or crusts, and can become infected. In some cases these blisters can cover large areas of the skin.

Bullous pemphigoid is a long-term debilitating disease because of the long-term blistering, itching and skin damage which can lead to infection. Suppression of the immune system by the medicines needed to treat the condition can be life threatening.

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

At the time of designation, bullous pemphigoid affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 129,000 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 patients with bullous pemphigoid were treated with medicines to reduce inflammation, in particular corticosteroids. The corticosteroid prednisolone was authorised in some EU

^{*}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 517,400,000 (Eurostat 2018).



countries for the treatment of bullous pemphigoid. Patients were also given medicines to suppress the immune system, such as azathioprine, to allow lower doses of corticosteroids to be used.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with bullous pemphigoid because early data indicate that patients that use the medicine may be able to reduce their use of corticosteroids.

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?

Bertilimumab is a monoclonal antibody (a type of protein) which is designed to attach to eotaxin-1 and block its activity. Eotaxin-1 is a substance that is involved in inflammation in patients with bullous pemphigoid. By blocking its action, bertilimumab reduces inflammation and symptoms associated with the disease.

What is the stage of development of this medicine?

The effects of bertilimumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with bertilimumab in patients with bullous pemphigoid were ongoing.

At the time of submission, bertilimumab was not authorised anywhere in the EU for bullous pemphigoid 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 19 July 2018 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	Bertilimumab	Treatment of bullous pemphigoid
Bulgarian	Бертилимумаб	Лечение на булозен пемфигоид
Croatian	Bertilimumab	Liječenje buloznog pemfigoida
Czech	Bertilimumab	Léčba bulózního pemfigoidu
Danish	Bertilimumab	Behandling af bulløs pemfigoid
Dutch	Bertilimumab	Behandeling van parapemphigus
Estonian	Bertilimumab	Põieendpemfigoidi ravi
Finnish	Bertilimumabi	Rakkulaisen pemfigoidin hoito
French	Bertilimumab	Traitement de la pemphigoïde bulleuse
German	Bertilimumab	Behandlung von bullösem Pemphigoid
Greek	Μπερτιλιμουμάμπη	Θεραπεία της πομφολυγώδους πεμφιγοειδούς
Hungarian	Bertilimumab	Bullózus pemfigoid kezelése
Italian	Bertilimumab	Trattamento del pemfigoide bolloso
Latvian	Bertilimumabs	Bullozā pemfigoīda ārstēšana
Lithuanian	Bertilimumabas	Pūslinio pemfigoido gydymas
Maltese	Bertilimumab	Kura tal-pemfigojde bl-infafet
Polish	Bertilimumab	Leczenie pęcherzowego pemfigoidu
Portuguese	Bertilimumab	Tratamento do penfigoide bolhoso
Romanian	Bertilimumab	Tratamentul pemfigoidului bulos
Slovak	Bertilimumab	Liečba bulózneho pemfigoidu
Slovenian	Bertilimumab	Zdravljenje buloznega pemfigoida
Spanish	Bertilimumab	Tratamiento de penfigoide ampolloso
Swedish	Bertilimumab	Behandling av bullös pemfigoid
Norwegian	Bertilimumab	Behandling av bulløs pemfigoid
Icelandic	Bertilímúmaþ	Meðferð á blöðrumyndandi pemfigus

¹ At the time of designation