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Public summary of opinion on orphan designation

Nomacopan for the treatment of bullous pemphigoid

On 26 June 2020, orphan designation EU/3/20/2289 was granted by the European Commission to Akari Malta Limited, Malta, for Nomacopan (also known as Coversin) 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 body's natural defences 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 become infected. In some cases, these blisters can cover large areas of the skin.

Bullous pemphigoid is a long-lasting 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 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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).

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



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 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 whose condition did not improve with corticosteroids alone improved after addition of nomacopan. The effects seen were also maintained after stopping 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?

Nomacopan attaches to two molecules, complement protein C5 and leukotriene B4 (LTB4), which are activated or produced as part of the inflammatory response that causes blisters in bullous pemphigoid. By binding to these molecules and blocking their activity, nomacopan is expected to reduce or slow down the damage to skin caused by inflammation, thereby improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of nomacopan have been evaluated in experimental models.

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

At the time of submission, nomacopan was not authorised anywhere in the EU for the treatment of bullous pemphigoid. Orphan designation of nomacopan had been granted in the United States for bullous pemphigoid.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 May 2020, 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

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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	Nomacopan	Treatment of bullous pemphigoid
Bulgarian	Номакопан	Лечение на булозен пемфигоид
Croatian	Nomakopan	Liječenje buloznog pemfigoida
Czech	Nomacopan	Léčba bulózního pemfigoidu
Danish	Nomacopan	Behandling af bulløs pemfigoid
Dutch	Nomacopan	Behandeling van parapemphigus
Estonian	Nomakopaan	Põieendpemfigoidi ravi
Finnish	Nomakopaani	Rakkulaisen pemfigoidin hoito
French	Nomacopan	Traitement de la pemphigoïde bulleuse
German	Nomacopan	Behandlung von bullösem Pemphigoid
Greek	Νομακοπάνη	Θεραπεία της του πομφολυγώδους πεμφιγοειδούς
Hungarian	Nomakopan	Bullózus pemfigoid kezelése
Italian	Nomacopan	Trattamento del pemfigoide bolloso
Latvian	Nomakopāns	Bullozā pemfigoīda ārstēšana
Lithuanian	Nomakopanas	Pūslinio pemfigoido gydymas
Maltese	Nomacopan	Kura tal-pemfigojde bl-infafet
Polish	Nomakopan	Leczenie pęcherzowego pemfigoidu
Portuguese	Nomacopano	Tratamento do penfigoide bolhoso
Romanian	Nomacopan	Tratamentul pemfigoidului bulos
Slovak	Nomakopan	Liečba bulózneho pemfigoidu
Slovenian	Nomakopan	Zdravljenje buloznega pemfigoida
Spanish	Nomacopano	Tratamiento de penfigoide ampolloso
Swedish	Nomakopan	Behandling av bullös pemfigoid
Norwegian	Nomakopan	Behandling av bulløs pemfigoid
Icelandic	Nomacopan	Meðferð á blöðrumyndandi pemfigus

¹ At the time of designation