

15 January 2019
EMA/770508/2018

Public summary of opinion on orphan designation

Imlifidase for the treatment of anti-glomerular basement membrane disease

On 19 November 2018, orphan designation (EU/3/18/2096) was granted by the European Commission to Hansa Medical AB, Sweden, for imlifidase for the treatment of anti-glomerular basement membrane disease.

What is anti-glomerular basement membrane disease?

Anti-glomerular basement membrane disease is a disease in which the immune system (the body's natural defences) produces abnormal antibodies against targets present in the glomerular basement membrane (GBM) found in the kidneys and the alveolar basement membrane in the lungs.

Patients with anti-GBM disease develop inflammation and bleeding in the lung and a kidney disease called glomerulonephritis, in which the small filtering units in the kidneys are damaged.

Anti-GBM disease is a long-term debilitating and life-threatening disease that can lead to lung problems and kidney failure.

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

At the time of orphan designation, anti-GBM disease affected approximately 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of around 1,000 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 orphan designation, treatments for anti-GBM disease included medicines to reduce inflammation, such as corticosteroids and cyclophosphamide, as well as plasma exchange, a procedure to remove antibodies from plasma (the liquid part of the blood) by replacing the patient's plasma with plasma from a donor.

*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).

The sponsor has provided sufficient information to show that imlifidase might be of significant benefit for patients with anti-GBM disease because preliminary data showed that patients treated with the medicine had improved kidney function compared with standard of care.

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?

Imlifidase is made of an enzyme derived from the bacterium *Streptococcus pyogenes*. This enzyme breaks down certain types of antibodies including the abnormal antibodies produced in anti-GBM disease. By breaking down the abnormal antibodies, the medicine is expected to reduce inflammation and improve 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 anti-GBM disease were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for anti-GBM disease. Orphan designation of the medicine had been granted in the United States for anti-GBM disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 October 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	Imlifidase	Treatment of anti-glomerular basement membrane disease
Bulgarian	Имлифидаза	Лечение на болест на анти-гломерулна базална мембрана
Croatian	Imiflidaza	Liječenje bolesti protutijela na bazalnu membranu glomerula
Czech	Imflidasa	Léčba onemocnění anti-glomerulární basální membrány
Danish	Imlifidase	Behandling af anti-glomerulær basement membra sygdom (Goodpastures syndrom)
Dutch	Imlifidase	Behandeling van anti-glomulair basaal membraan ziekte
Estonian	Imlifidaas	Anti-glomerulaarse basaalmembraani haiguse ravi
Finnish	Imlifidaasi	Tyvikalvovasta-aineglomerulonefriitin hoito
French	Imlifidase	Traitement de la maladie de la membrane basale anti-glomérulaire
German	Imlifidase	Behandlung der Antikörper vermittelten Krankheit der glomerulären Basalmembran
Greek	Ιμλιφιδόση	Θεραπεία της νόσου από αντισώματα έναντι της βασικής μεμβράνης των νεφρικών σπειραμάτων
Hungarian	Imlifidáz	Antiglomeruláris bazálmembrán betegség kezelése
Italian	Imlifidase	Trattamento della malattia della membrana basale anti-glomerulare
Latvian	Imlifidāze	Anti-glomerulu bazālās membrānas slimības ārstēšana
Lithuanian	Imlifidazė	Antiglomerulo bazinės membranos ligos gydymas
Maltese	Imlifidase	Il-kura tal-marda tal-qiegħ tal-membrani antiglomerulari
Polish	Imlifidaza	Leczenie zespołu płucno-nerkowego
Portuguese	Imlifidase	Tratamento da doença anti-membrana basal glomerular
Romanian	Imiflidază	Tratamentul bolii anti-membrană bazală glomerulară
Slovak	Imflimidáza	Liečba choroby anti-glomerulárnej bazálnej membrány
Slovenian	Imlifidaza	Zdravljenje bolezni protiteles proti glomerulni bazalni membrani
Spanish	Imlifidase	Tratamiento de la enfermedad de la membrana basal anti-glomerulario
Swedish	Imlifidas	Behandling av antiglomerulär basalmembransjukdom
Norwegian	Imlifidase	Behandling av anti-glomerulær basalmembran sykdom
Icelandic	Imlifidasi	Meðferð við sjúkdómi sem stafar af mótefnum gegn grunnhimnu gaukla

¹ At the time of designation