



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Budesonide for the treatment of primary IgA nephropathy

On 18 November 2016, orphan designation (EU/3/16/1778) was granted by the European Commission to Pharmalink AB, Sweden, for budesonide for the treatment of primary IgA nephropathy.

What is primary IgA nephropathy?

Primary IgA nephropathy is a disease caused by the immune system (the body's natural defences) producing a faulty version of an antibody called immunoglobulin A (IgA), which builds up in small blood vessels in the kidney, called glomeruli, that filter the blood. This damages the glomeruli and makes them leak blood and protein into the urine.

Primary IgA nephropathy is a long-term debilitating and life-threatening disease because the kidneys gradually stop working properly and eventually fail, requiring dialysis or a kidney transplant.

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

At the time of designation, primary IgA nephropathy affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 205,000 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 submission of the application for orphan drug designation, no satisfactory methods were authorised for primary IgA nephropathy. Patients were often treated with medicines to suppress the immune system, such as corticosteroids, ciclosporin or cyclophosphamide. Patients at risk of developing kidney failure were given medicines to lower blood pressure, such as ACE inhibitors or angiotensin receptor blockers, to reduce stress on the kidneys. As the disease worsens, kidney dialysis and kidney transplant may be needed.

*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 513,700,000 (Eurostat 2016).



How is this medicine expected to work?

Budesonide is a well-known type of medicine called a corticosteroid. Corticosteroids have a wide range of effects that suppress the immune system. This medicine is designed to be released when it reaches the intestine, where it is expected to reduce the production of faulty IgA antibodies from immune tissue in the intestine. This is expected to decrease their build-up in the kidneys and so reduce damage to the kidneys.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials with budesonide in patients with primary IgA nephropathy were ongoing.

At the time of submission, budesonide was authorised in the EU for treatment of inflammatory conditions such as asthma, Crohn's disease and ulcerative colitis.

At the time of submission, budesonide was not authorised anywhere in the EU for primary IgA nephropathy. Orphan designation of budesonide had been granted in the US for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 October 2016 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	Budesonide	Treatment of primary IgA nephropathy
Bulgarian	Будезонид	Лечение на първична IgA нефропатия
Croatian	Budezonid	Liječenje primarne IgA nefropatije
Czech	Budesonid	Léčba primární IgA nefropatie
Danish	Budesonid	Behandling af primære IgA-nefropati
Dutch	Budesonide	Behandeling van primaire IgA nefropathie
Estonian	Budsoniid	Primaarse IgA nefropaatia ravi
Finnish	Budesonidi	Primaarisen IgA-nefropatian hoito
French	Budésonide	Traitement de la néphropathie primaire à IgA
German	Budesonid	Behandlung der primären IgA-Nephropathie
Greek	βουδεσονίδη	Θεραπεία της πρωτογενούς IgA νεφροπάθειας
Hungarian	Budezonid	Primér IgA nefropátia kezelése
Italian	Budesonide	Tattamento della nefropatia da IgA
Latvian	Budezonīds	Primāras IgA nefropātijas ārstēšana
Lithuanian	Budezonidas	Pirminės IgA nefropatijos gydymas
Maltese	Budesonide	Il-kura ta' IgA nefropatija primarja
Polish	Budezonid	Leczenie pierwotnej nefropatii IgA
Portuguese	Budesonida	Tratamento da nefropatia primária por IgA
Romanian	Budesonidă	Tratamentul nefropatiei primare cu IgA
Slovak	Budezonid	Liečba primárnej IgA nefropatie
Slovenian	Budezonid	Zdravljenje primarne IgA nefropatije
Spanish	Budesónida	Tratamiento de la nefropatía por IgA primaria
Swedish	Budesonid	Behandling av primär IgA nefropati
Norwegian	Budesonid	Behandling av primær IgA nefropati
Icelandic	Búdesóníð	Meðferð frumkomins IgA nýrnasjúkdóms

¹ At the time of designation