

6 April 2018
EMA/64972/2018

Public summary of opinion on orphan designation

Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 for the treatment of primary IgA nephropathy

On 22 February 2018, orphan designation (EU/3/18/1984) was granted by the European Commission to Omeros London Limited, United Kingdom, for recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 (also known as OMS721) 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 build-up damages the glomeruli, causing leakage of 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 207,000 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, no satisfactory methods were authorised for treating primary IgA nephropathy. Patients were treated with ACE inhibitors or angiotensin receptor blockers to lower blood pressure, and with medicines to suppress the immune system, such as corticosteroids, ciclosporin or cyclophosphamide. 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 517,400,000 (Eurostat 2018).

How is this medicine expected to work?

This medicine is made up of a monoclonal antibody (a type of protein) that has been designed to attach to and block an enzyme called mannan-binding lectin serine protease 2 (MASP-2). This enzyme is involved in activating proteins in the 'complement system', which is part of the immune system. The complement system plays a role in inflammation of the kidneys in primary IgA nephropathy. By blocking MASP-2, the medicine is expected to reduce the immune response that causes 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 primary IgA nephropathy were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for primary IgA nephropathy. Orphan designation of the medicine had been granted in the United States for primary IgA nephropathy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 January 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	Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2	Treatment of primary IgA nephropathy
Bulgarian	Рекомбинантно моноклонално антитяло срещу манан-свързваща, лектин-асоциирана серин протеаза-2	Лечение на първична IgA нефропатия
Croatian	Rekombinantno ljudsko monoklonsko protutijelo protiv manan-vezajuće serin proteaze 2 povezane s lektinom	Liječenje primarne IgA nefropatije
Czech	Rekombinantní humánní monoklonální protilátka proti mannan-vázajícího lektinu asociovaného se serinovou proteázou-2	Léčba primární IgA nefropatie
Danish	Rekombinant human monoklonal antistof mod mannan-bindende lektin-associeret serinprotease-2	Behandling af primære IgA-nefropati
Dutch	Recombinant humaan monoclonaal antilichaam gericht tegen mannan-bindend lectine-geassocieerd serine protease-2	Behandeling van primaire IgA nefropathie
Estonian	Mannan-siduva lektiiniga seotud seriini proteaas 2 vastane rekombinantne inimese monoklonaalne antikeha	Primaarse IgA nefropaatia ravi
Finnish	Rekombinantti ihmisen monoklonaalinen vasta-aine mannaaniin sitoutuvaa, lektiiniin assosioutuvaa proteaasi 2:ta vastaan.	Primaarisen IgA-nefropatian hoito
French	Anticorps monoclonal humain recombinant dirigé contre la sérine protéase-2 associée à la lectine liant le mannose	Traitement de la néphropathie primaire à IgA
German	Rekombinanter humaner monoklonaler Antikörper gegen Mannan-Binding Lectin-Associated Serine Protease 2	Behandlung der primären IgA-Nephropathie
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι της προσδένουσας σε μαννάνη σχετιζόμενης με λεκτίνη πρωτεάσης σερίνης-2	Θεραπεία της πρωτογενούς IgA νεφροπάθειας
Hungarian	Mannan-kötő lektin-asszociált szerin proteáz-2 elleni rekombináns humán monoklonális antitest	Primér IgA nefropátia kezelése
Italian	Anticorpo monoclonale umano ricombinante contro la serina proteasi-2 associata a la lectina che lega il mannoso	Trattamento della nefropatia da IgA
Latvian	Rekombinanta cilvēka monoklonālā antivielā, kas vērsta pret mannān-saistošu lecitīn-asociētu serīna proteāzi-2	Primāras IgA nefropātijas ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš mananą surišančią su lektinu siejamą serino proteazę 2	Pirminės IgA nefropatijos gydymas

¹ At the time of designation

Language	Active ingredient	Indication
Maltese	Antikorp monoklonali uman rikombinanti kontra proteaži-2 serina assoċjata ma' lektina u li jeħel ma' mannan	Il-kura ta' IgA nefropatija primarja
Polish	Recombinowane ludzkie przeciwciało monoklonalne przeciwko proteazie serynowej-2 wiążącej mannan i skojarzonej z lektyną	Leczenie pierwotnej nefropatii IgA
Portuguese	Anticorpo monoclonal humano recombinante contra a serina protease-2 associada à lectina de ligação à manose	Tratamento da nefropatia primária por IgA
Romanian	Anticorup uman monoclonal recombinant împotriva serin proteazei-2 asociate cu MBL ("mannan-binding lectin")	Tratamentul nefropatiei primare cu IgA
Slovak	Rekombinantná ľudská monoklonálna protilátka proti mannan-viažucej lektín-asociovej serín proteáze-2	Liečba primárnej IgA nefropatie
Slovenian	Recombinantno humano monoklonsko protitelo proti manozo vezajoči, z lektinom povezani serinski proteazi-2	Zdravljenje primarne IgA nefropatije
Spanish	Anitcuerpo humano monoclonal recombinante contra la protease 2 serina asociada a la lectina liada a manan.	Tratamiento de la nefropatía por IgA primaria
Swedish	Rekombinant human monoklonal antrikropp mot mannan-bindande lektin-associerat serinproteas-2	Behandling av primär IgA nefropati
Norwegian	Rekombinant humant monoklonalt antistoff mot mannanbindende lektinassosiert serinprotease-2	Behandling av primær IgA nefropati
Icelandic	Raðbrigða manna einstofna mótefni gegn mannan-bindandi lektín-tengdur serín próteasi-2	Meðferð frumkomins IgA nýrnasjúkdóms