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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human minibody against complement component C5 for the treatment of primary membranoproliferative glomerulonephritis

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Rev.1: sponsor's name and address change	20 January 2014

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 27 October 2011, orphan designation (EU/3/11/926) was granted by the European Commission to Adienne S.r.l, Italy, for recombinant human minibody against complement component C5 for the treatment of primary membranoproliferative glomerulonephritis.

In January 2014, ADIENNE S.r.l. changed name to ADIENNE S.r.l.S.U.

What is primary membranoproliferative glomerulonephritis?

Primary membranoproliferative glomerulonephritis is a kidney disease in which parts of the glomeruli, (small filtering units in the kidneys which filter waste products from the blood and start urine production), are damaged.

The damage is caused by the body's immune system attacking the glomeruli which leads to impairment of blood filtering, causing leakage of protein into the urine. Some patients have swelling in various parts of their body due to the large amount of protein that has leaked into the urine (nephrotic syndrome) while others may develop high blood pressure and have blood and protein in the urine (nephritic syndrome).

Primary membranoproliferative glomerulonephritis is a debilitating disease that is long lasting and may be life threatening because it can lead to nephrotic syndrome and complete failure of kidney function (end-stage kidney disease).

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

At the time of designation, primary membranoproliferative glomerulonephritis affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 81,000 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 there were no satisfactory treatments authorised in the EU for this condition. Because the disease involves the body's immune system, immunosuppressant medicines were usually used. Other treatments used include plasmapheresis (a blood purification procedure), plasma infusion and antiplatelet agents. Patients developing end-stage kidney disease received dialysis and kidney transplantation.

How is this medicine expected to work?

The part of the immune system involved in damaging the glomeruli is called the complement system, which is a group of proteins in the blood that can be activated to attack cells recognised as 'foreign'.

This medicine is a small antibody (minibody) designed to target and attach to an important protein in the complement system called C5. By attaching to C5, the medicine is expected to block the chain of reactions that damages the glomeruli.

The medicine is produced by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA), which makes it able to produce the medicine.

What is the stage of development of this medicine?

The effects of recombinant human minibody against complement component C5 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with primary membranoproliferative glomerulonephritis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for primary membranoproliferative glomerulonephritis 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 16 September 2011 recommending the granting of this designation.

[*]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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human minibody against complement component C5	Treatment of primary membranoproliferative glomerulonephritis
Bulgarian	Рекомбинантно човешко минитяло срещу C5 компонента на комплемента	Лечение на първичен мембрано-пролиферативен гломерулонефрит.
Czech	Rekombinantní lidská miniprotilátka proti C5 složce komplementu	Léčba primární membranoproliferativní glomerulonefritidy
Danish	Rekombinant humant antistoffragment mod komplement komponent C5	Behandling af primær membranoproliferativ glomerulonefritis
Dutch	Recombinant humaan minilichaam gericht tegen complement component C5	Behandeling van primaire membranoproliferatieve glomerulonefritis
Estonian	Komplemendi osa C5 vastane rekombinantne inimese minikeha	Primaarse membranoosproliferatiivse glomerulonefriidi ravi
Finnish	Rekombinantti humaani minivasta-aine komplementti komponentti C5:a vastaan	Primaarisen membranoproliferatiivisen glomerulonefriitin hoito
French	Minibody humain recombinant dirigé contre l'élément C5 du complément	Glomerulonephrite membranoproliferative primaire
German	Humaner rekombinanter Minibody gegen den Komplementfaktor C5	Behandlung der primären, membranoproliferativen Glomerulonephritis
Greek	Ανασυνδιασμενο ανθρώπινο μινίσωμα ενάντια στον παράγοντα C5 του συμπληρώματος	Θεραπεία πρωτογενούς μεμβρανοϋπερπλαστικής σπειραματονεφρίτιδας
Hungarian	C5 komplement-komponens ellenes rekombináns humán minitest	Primér membránproliferatív glomerulonefritisz kezelése
Italian	Minianticorpo umano ricombinante diretto contro la componente C5 del complemento	Trattamento della glomerulonefrite membranoproliferativa primaria
Latvian	Rekombinantas cilvēka minivielas pret komplementa C5 komponenti	Primāra membranoproliferatīva glomerulonefrīta ārstēšana
Lithuanian	Rekombinantinis žmogaus minikūnelis prieš komplemento komponentą C5	Pirminio membranoproliferacinio glomerulonefrito gydymas
Maltese	Minikorp uman rikombinanti kontra l-komponent Ċ5 tal-komplement	Kura tal-glomerulonefrite membranoproliferattiva primarja
Polish	Rekombinowane ludzkie miniciało przeciwko składowej dopełniacza C5	Leczenie pierwotnego błoniasto-rozplemowego kłębuszkowego zapalenia nerek

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Minicorpo recombinante de origem humana contra o componente C5 do complemento	Tatamento da glomerulonefrite membranoproliferativa primária
Romanian	Minianticorp uman recombinant anti componenta C5 a complementului	Tratamentul glomerulonefritei membranoproliferative primare
Slovak	Rekombinantná ľudská miniprotilátka proti zložke komplementu C5	Liečba primárnej membránoproliferatívnej glomerulonefritídy
Slovenian	Rekombinantno humano minitelo proti sestavini C5 komplementa	Zdravljenje primarnega membranskega proliferativnega glomerulonefritisa
Spanish	Minianticuerpo recombinante humano contra el factor C5 del complemento	Tratamiento de la glomerulonefritis membranoproliferativa primaria
Swedish	Recombinant human minikropp riktat mot komplement komponenten C5	Behandling av primär membranoproliferativ glomerulonefrit
Norwegian	Rekombinant humant antistoffragment mot komplementfaktor C5	Behandling av primær membranoproliferativ glomerulonefritt
Icelandic	Raðbrigða manna minibody gegn komplementþætti C5	Meðferð á membranópróliferatífri gauklabólgu