



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human normal immunoglobulin for treatment in organ transplantation

On 20 April 2017, orphan designation (EU/3/17/1866) was granted by the European Commission to Hôpital Foch, France, for human normal immunoglobulin for treatment in solid organ transplantation.

What is solid organ transplantation?

Solid organ transplantation is a surgical procedure in which a diseased organ, such as the heart, lungs, liver or kidney, is replaced with an organ from a donor.

Transplantation is a very complex procedure. During transplantation, the organ to be transplanted can become damaged because of the interruption and restoration of blood supply to the organ. In addition, graft rejection can occur after transplantation, when the recipient's body rejects the transplanted organ. Graft rejection is caused by the patient's immune system (the body's natural defences) recognising the transplanted graft as 'foreign' and attacking it.

These complications can be debilitating and life-threatening because they may result in the transplanted organ not working properly.

What is the estimated number of patients affected by graft rejection following solid organ transplants?

At the time of designation, approximately 1 in 10,000 people per year in the European Union (EU) have solid organ transplants. This was equivalent to a total of around 52,000 people per year^{*}, and is below the ceiling for orphan designation. 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, several medicines were authorised in the EU to treat graft rejection in solid organ transplantation. These include antibodies such as antilymphocyte immunoglobulin and thymoglobulin, medicines that suppress the immune process such as azathioprine, ciclosporin,

^{*}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 (European Union (EU 28), Norway, Iceland and Liechtenstein). This represents a population of 515,700,000 (Eurostat 2017).



mycophenolate mofetil and tacrolimus, and corticosteroids such as prednisolone or methylprednisolone.

The sponsor has provided sufficient information to show that human normal immunoglobulin might be of significant benefit for patients undergoing solid organ transplantation because published studies indicate that this medicine may improve survival of the transplanted organ. 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?

Immunoglobulins, also called antibodies, are proteins naturally found in the blood that help the body to fight diseases. They are used for managing a number of different conditions. This medicine contains a mixture of antibodies taken from the blood of many healthy donors. The medicine will be given to patients who have undergone a transplant and is expected to prevent the patient's antibodies from attacking the transplanted organ. This is expected to decrease organ damage and lower the risk of the transplant not working.

What is the stage of development of this medicine?

The effects of this medicine have not been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with this medicine in patients undergoing solid organ transplantation was planned.

At the time of submission, this medicine was not authorised anywhere in the EU for solid organ transplantation 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 15 March 2017 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	Human normal immunoglobulin	Treatment in solid organ transplantation
Bulgarian	Нормален човешки имуноглобулин	Лечение при трансплантация на солиден орган
Croatian	Humani normalni imunoglobulin	Liječenje u transplantaciji solidnih organa
Czech	Normální lidský imunoglobulin	Léčba pro transplantaci solidních orgánů
Danish	Humant normalt immunoglobulin	Behandling i organ transplantation
Dutch	Humaan normaal immunoglobuline	Behandeling bij soliede orgaantransplantatie
Estonian	Inimese normaalne immunoglobuliin	Kasutamiseks elundite siirdamise ravis
Finnish	Ihmisen normaali immunoglobuliini	Hoito kiinteän elimen siirron yhteydessä
French	Immunoglobuline humaine normale	Traitement pour la transplantation d'organes solides
German	Normales Immunglobulin vom Menschen	Behandlung in Organtransplantation
Greek	Ανθρώπινη φυσιολογική ανοσοσφαιρίνη	Θεραπεία στη μεταμόσχευση συμπαγών οργάνων
Hungarian	Humán normál immunglobulin	Szervtranszplantáció esetén alkalmazandó
Italian	Immunoglobulina umana normale	Trattamento nel trapianto di organi solidi
Latvian	Cilvēka normālais imūnglobulīns	Ārstēšanai norobežoto orgānu transplantācijā
Lithuanian	Žmogaus normalus imunoglobulinas	Transplantacijos parenchiminiame organe gydymas
Maltese	Immunoglobulina normali umana	Kura fi trapjant ta' organi solidi
Polish	Immunoglobulina ludzka normalna	Leczenie w przebiegu transplantacji organów
Portuguese	Imunoglobulina humana normal	Tratamento em transplante de órgãos sólidos
Romanian	Imunoglobulină umană normală	Tratament în transplantul de organe solide
Slovak	Normálny ľudský imunoglobulín	Liečba pri transplantácii celého orgánu
Slovenian	Humani normalni imunoglobulin	Zdravljenje pri transplantaciji organov
Spanish	Inmunoglobulina humana normal	Tratamiento del trasplante de órgano sólido
Swedish	Humant normalt immunoglobulin	Behandling vid organtransplantation.
Norwegian	Humant normalt immunoglobulin	Behandling ved transplantasjon av solide organer
Icelandic	Eðlilegt manna ímmúnóglóbúlín	Meðferð við líffæraígræðslu

¹ At the time of designation