5 August 2020
EMADOC-628903358-2225

Public summary of opinion on orphan designation

Anti-(integrin beta-3) human monoclonal antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility

On 22 April 2020, orphan designation EU/3/20/2273 was granted by the European Commission to FGK Representative Service GmbH, Germany, for anti-(integrin beta-3) human monoclonal antibody (also known as RB 212) for the prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility.

What is fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility?

Fetal and neonatal alloimmune thrombocytopenia (FNAIT) due to human platelet antigen-1a incompatibility is a rare disease in which fetuses and newborn babies have low levels of platelets (components of the blood involved in clotting).

It occurs when the mother produces antibodies that cross the placenta to attack the baby's platelets because the platelets contain a substance (human platelet antigen-1a (HPA-1a)), which the mother's immune system recognises as foreign.

FNAIT is chronically debilitating and life threatening since it can cause bleeding within the skull in the fetus or newborn baby, and can lead to miscarriage, stillbirth, death of the newborn baby or permanent damage to the child's brain and nerves.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of FNAIT due to human platelet antigen-1a incompatibility was estimated to be approximately 2.5 people in 10,000 in the European Union (EU). This was equivalent to a total of around 130,000 people*, and is below the ceiling for orphan

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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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 in the EU for the prevention of FNAIT due to human platelet antigen-1a incompatibility.

How is this medicine expected to work?

The medicine contains a monoclonal antibody (a type of protein) designed to attach to a specific target, in this case HPA-1a. Unlike antibodies produced by the mother, when the medicine is injected into the mother's blood, it does not pass the placenta and therefore does not affect the fetus. It is expected to work by attaching to and removing any HPA-1a platelets produced by the fetus that enter the mother's circulation, and preventing the mother's immune system from reacting against them. In this way the immune system reaction from the mother's body against fetal platelets can be avoided, thus preventing the occurrence of the condition.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with FNAIT due to human platelet antigen-1a incompatibility had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the prevention of FNAIT due to human platelet antigen-1a incompatibility or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 19 March 2020, 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.

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	Anti-(integrin beta-3) human monoclonal antibody	Prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility
Bulgarian	Анти (интегрин бета 3) човешко моноклонално антитяло	Профилактика на фетална и неонатална алоимунна тромбоцитопения поради несъвместимост на човешки тромбоцитен 1a антиген
Croatian	Anti- (integrin beta-3) humano monoklonsko protutijelo	Prevencija fetalne i neonatalne aloimune trombocitopenije zbog nekompatibilnosti humanog trombocitnog antigena-1a
Czech	Lidská monoklonální protilátka proti beta-3 integrinu	Prevence fetální a neonatální aloimunitní trombocytopenie způsobené inkompatibilitou lidského destičkového antigenu-1a
Danish	Anti -(integrin beta-3) human monoklonal antistof	Forebyggelse af føtal og neonatal alloimmun thrombocytopeni grundet thrombocyt antigen-1a uforligelighed
Dutch	Anti-(integrin beta-3) humaan monoclonaal antilichaam	Preventie van fetale en neonatale alloimmune thrombocytopenia ten gevolge van humane bloedplaatjesantigen-1a incompatibiliteit
Estonian	Beeta-3-integriini vastane inimese monoklonaalne antikeha	Inimese trombotsüütide antigeen-1apuudulikkusest tingitud looteea ja neonataalse alloimmuunse trombotsütopeenia ennetus
Finnish	Ihmisen monoklonaalinen vasta-aine beeta3-integriiniä vastaan	Äidin ja sikiön verihiutaleantigeenin 1a yhteensopimattomuudesta johtuvan fetaalin ja neonataalisen alloimmuunin trombosytopenian ehkäisy
French	Anticorps humain monoclonal anti-integrine beta-3	Prévention de la thrombocytopénie alloimmune foetale et neonatale due à une incompatibilité liée à l'antigène plaquettaire humain 1a
German	Anti-(integrin beta-3) humaner monoklonaler Antikörper	Prävention fötaler und neonataler Alloimmun-Thrombozytopenie infolge humaner Plättchen Antgen-1a Inkompatibilität
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα έναντι β3-ιντεγκρίνης	Πρόληψη της εμβυϊκής και εμβρυϊκής αλλοάνοσης θρομβοκυτταροπενίας λόγω ασυμβατότητας ανθρώπινου αντιγόνου-1a αιμοπεταλίων
Hungarian	Integrin beta-3 ellenes human monoklonális antitest	Humán vérlemezke antigen-1a inkompatibilitás okozta foetalis és neonatális alloimmune trombocítopénia megelőzése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	Anticorpo monoclonale umano antii integrina beta 3	Prevezione della trombocitemia alloimmune fetale e neonatale causata dall'incompatibilita'all'antigene 1a delle piastrine umane.
Latvian	Cilvēka monoklonāla antiviela pret bēta-3 integrīnu	Cilvēka trombocītu antigēna-1a nesaderības izraisītas augļa un jaundzimušā alloimūnas trombocitopēnijas prevencija
Lithuanian	Anti-(integrin beta-3) žmogaus monokloninis antikūnas	Vaisiaus ir naujagimių aloimuninės trombocitopenijos prevencija dėl žmogaus trombocitų antigeno 1a nesuderinamumo
Maltese	Antikorp monoklonali tal-bniedem anti-(integrin beta-3)	Prevenzjoni ta' tromboċitopenija alloimmuni fil-fetu u f'tarbija tat-twelid minħabba inkompatibbiltà ta' antiġene-1a tal-pjastrini tal-bniedem
Polish	Ludzkie monoklonalne przeciwciało nakierowane przeciw integrynie beta 3	Zapobieganie alloimmunizacyjnej małopłytkowości płodowej i noworodkowej z powodu niezgodności ludzkiego antygenu płytkowego 1a
Portuguese	Anticorpo monoclonal humano anti-(integrina beta-3)	Prevenção da trombocitopenia aloimune fetal e neonatal devido à incompatibilidade para o antigénio plaquetário humano 1a
Romanian	Anticorp monoclonal uman anti-(integrin beta-3)	Prevenția trombocitopeniei aloimune fetale si neonatale datorată incompatibilitatii antigenului 1-a plachetar uman
Slovak	Anti-(integrin beta-3) ľudská monoklonálna protilátka	Prevencia fetálnej a neonatálnej aloimunitnej trombocytopénie v dôsledku nekompatibility ľudského doštičkového antigénu-1a
Slovenian	Humano monoklonsko protitelo proti integrinu beta-3	Preprečevanje fetalne in neonatalne aloimunske trombocitopenije zaradi antigen-1a inkompatibilnosti humanih trombocitov
Spanish	Anticuerpo monoclonal humano anti integrino beta-3	Prevencion de la trombocitopenai aloimune fetal y neonatal provocado por la incompatibildad al anigeno-1a human
Swedish	Anti-(integrin beta-3) human monoklonal antikropp	Förebyggande av fetal neonatal alloimmun trombocytopeni pga. humant blodplättsantigen-1a inkompatibilitet
Norwegian	Anti-(integrin beta-3) humant monoklonalt antistoff	Forebygging av føtal og neonatal alloimmun trombocytopeni (FNAIT) som skyldes uforlikelighet i humant plateantigen-1a (HPA-1a)
Icelandic	And-(integrin beta-3) einstofna mótefni manna	Fyrirbyggjandi meðferð við samónæmis blóðflagnafæð í fóstrum og nýburum sem stafar af ósamrýmanleika við mótefnavaka-1a á blóðflögum manna