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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human platelet antigen-1a immunoglobulin for the prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility

On 27 October 2011, orphan designation (EU/3/11/922) was granted by the European Commission to Prophylix Pharma AS, Norway, for human platelet antigen-1a immunoglobulin 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 of foetuses and newborn babies. It occurs when the foetus' platelets (a type of blood cell) produce certain antigens (structures that a body can recognise as 'foreign') which are not normally found in the mother's body. These antigens are called 'human platelet antigen-1a' (HPA-1a). If they enter the mother's blood, then the mother's immune system responds by producing antibodies (proteins in the blood that help the body fight infections and diseases). These antibodies attack the blood platelets in the foetus, causing thrombocytopenia (low blood platelet counts). Because platelets are involved in blood clotting, the foetus or newborn baby is at risk of severe bleeding.

FNAIT is chronically debilitating and life threatening since it can lead to intracranial haemorrhage (bleeding within the skull) in the foetus or newborn baby, which can cause miscarriage, still birth, death of the newborn baby or permanent damage to the 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 fetal and neonatal alloimmune thrombocytopenia due to HPA-1a incompatibility was estimated to be approximately 2.8 people in 10,000 in the European Union (EU)*. This is equivalent to a total of 142,000 people, which is below

*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. This represents a population of 506,300,000 (Eurostat 2011).

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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 methods of prevention are available?

At the time of designation, no satisfactory methods were authorised in the EU for the prevention of FNAIT due to HPA-1a incompatibility.

How is this medicine expected to work?

The medicine consists of anti-HPA-1a immunoglobulins, taken from the blood of a suitable female donor containing antibodies against HPA-1a. Immunoglbulins are antibodies that attack certain antigens, in this case HPA-1a. When the medicine is injected into the mother's blood, it is expected to work by destroying the HPA-1a platelets produced by the foetus before the mother's immune system reacts against them. In this way the immune system reaction from the mother's body against the foetus platelets can be avoided, thus preventing the occurrence of fetal or neonatal alloimmune thrombocytopenia due to HPA-1a incompatibility.

What is the stage of development of this medicine?

The effects of HPA-1a immunoglobulin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with HPA-1a immunoglobulin had been started.

At the time of submission, HPA-1a immunoglobulin was not authorised anywhere in the EU for fetal and neonatal alloimmune thrombocytopenia due to HPA-1a incompatibility or designated as an orphan medicinal product anywhere in the world for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2011 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:

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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	Human platelet antigen-1a immunoglobulin	Prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility
Bulgarian	Човешки тромбоцити антиген-1а имуноглобулин	Предотвратяване на фетална и неонатална тромбоцитопения, дължаща се човешки тромбоцитен антиген- 1анесъвместимост
Czech	Lidský trombocytární antigen-1a imunoglobulin	Prevence fetální a neonatální aloimunní trombocytopenie z důvodů inkompatibility lidského trombocytárního antigenu-1a HPA-1a antigen neslučitelnost
Danish	Human trombocyt antigen-1a immunoglobulin	Forebyggelse af føtal og neonatal alloimmun trombocytopeni på grund af humant blodplade-antigen uforenelighed
Dutch	Menselijke bloedplaatjes antigen-1a immunoglobuline	Preventie van foetale en neonatale alloimmune trombocytopenie ten gevolge van humaan plaatjes antigen-1a incompatibiliteit
Estonian	Inimese trombotsüütide antigeeni-1a immunoglobuliin	Inimese trombotsüütide antigeeni- 1a sobimatusest tingitud loote ja vastsündinu alloimmuunse trombotsütopeenia ennetamine
Finnish	Ihmisen immunoglobuliini verihiutaleiden antigeeni-1a	Sikiön ja vastasyntyneen alloimmuunin trombosytopenian, joka johtuu verihiutaleiden antigeeni-1a:n yhteensopimattomuudesta, ehkäisy
French	Iimmunoglobulines anti-antigène plaquettaire humain 1-1	Prévention de la thrombopénie allo-immune du fœtus et du nouveau-né due à une incompatibilité d'antigène plaquettaire humain 1-a
German	Humanes Thrombozyten-Antigen-1a Immunglobulin	Prävention der durch eine Inkompatibilität von Humanem Thrombozyten-Antigen-1a verursachten fetalen und neonatalen Alloimmunthrombozytopenie
Greek	Ανοσοσφαιρίνη έναντι του αντιγόνου-1α των ανθρώπινων αιμοπεταλίων	Πρόληψη της εμβρύικής και νεογνικής αλλοάνοσης θρομβοπενίας λόγω ασυμβατότητας αντιγόνου-1α των ανθρώπινων αιμοπεταλίων
Hungarian	Humán thrombocyta antigén-1a immunglobulin	Humán thrombocyta antigén-1a összeférhetetlenség miatti magzati és újszülöttkori alloimmun thrombocytopénia
Italian	Immunoglobuline umane anti-antigene-trombocitario 1a (HPA-1a)	Prevenzione della trombocitopenia fetale e neonatale alloimmune da incompatibilità dell' antigene trobocitario 1a (HPA-1a)

[1] At the time of designation

Language	Active ingredient	Indication
Latvian	Cilvēka trombocītu antigēna-1a imunoglobulīns	Augļa un jaundzimušā alloimūnās trombocitopēnijas sakarā ar cilvēka trombocītu antigēna Ia nesaderību profilakse
Lithuanian	Žmogaus trombocitų antigeno-1a imunoglobulinas	Vaisiaus ar naujagimio aloimuninės trombocitopenijos, dėl žmogaus trombocitų antigeno – 1a nesuderinamumo, prevencija
Maltese	Immunoglobulina għall-antiġen tal-platelet uman 1a	Prevenzjoni tat-trombożitopenja alloimmuni fil-fetu u f'trabi tat-twelid minħabba inkompatibilità tal-antiġen tal-platelet uman 1a
Polish	Immunoglobulina przeciw antygenowi 1a ludzkich krwinek płytkowych	Zapobieganie, u płodów i noworodków, alloimmunologicznej trombocytopenii spowodowanej niezgodnością antygenu 1a ludzkich krwinek płytkowych
Portuguese	Imunoglobulina humana anti-antigénio-1a plaquetário	Prevenção da trombocitopénia aloimune fetal e neonatal causada por incompatibilidade ao antigénio-1a humano plaquetário
Romanian	Imunoglobuline anti-antigen plachetar uman-1a	Profilaxia trombocitopeniei aloimune fetale şi neonatale datorate unei incompatibilităţi de antigen plachetar uman-1a
Slovak	Imunoglobulín proti ľudskému doštičkovému antigénu 1a	Prevencia fetálnej a novorodeneckej aloimúnnej trombocytopénie v dôsledku inkompatibility ľudského doštičkového antigénu 1a
Slovenian	Imunoglobulin proti človeškemu trombocitnemu antigenu-1a	Preprečevanje fetalne in neonatalne aloimunske trombocitopenije zaradi nezdružljivosti s človeškim trombocitnim antigenom-1a
Spanish	Immunoglobulina humana anti antígeno humano plaquetario de tipo 1a	Prevención de la trombocitopenia aloimmune fetal y neonatal debida a la incompatibilidad del antígeno humano plaquetario de tipo 1a
Swedish	Humant trombocyt antigen-1a immunglobulin	Förebyggande av fetal och neonatal alloimmune trombocytopeni på grund av humant trombocyt antigen-1a inkompatibilitet
Norwegian	Humant blodplate antigen-1a immunglobulin	Forebygging av føtal og neonatal alloimmun trombocytopeni på grunn av humant blodplate antigen-1a uforlikelighet
Icelandic	Manna blóðflagna mótefnavaka-1a immúnóglóbúlíni	Fyrirbyggja fósturog nýbura sjálfsónæmis blóðflagnafæð vegna HPA-1a mótefnavaka ósamrýmanleika