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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

A highly purified formulation of *Staphylococcus aureus* protein A for the treatment of immune thrombocytopenia

On 9 October 2015, orphan designation (EU/3/15/1562) was granted by the European Commission to Coté Orphan Consulting UK Limited, United Kingdom, for a highly purified formulation of *Staphylococcus aureus* protein A for the treatment of immune thrombocytopenia.

What is immune thrombocytopenia?

Immune thrombocytopenia is an autoimmune condition in which the body's own immune system attacks the platelets in the blood that are needed for clotting. As a result, levels of platelet are low (thrombocytopenia) resulting in spontaneous bleeding and bruising. Although not all patients experience bleeding, severe bleeding can occur in some patients.

Immune thrombocytopenia is debilitating and life-threatening condition because of the risk of severe bleeding, especially in the brain.

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

At the time of designation, immune thrombocytopenia affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 180,000 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, authorised treatments available included human normal immunoglobulin, romiplostim and eltrombopag. Some patients required surgery (splenectomy) to remove the spleen, an organ involved in the removal of platelets from the body.

The sponsor has provided sufficient information to show that this highly purified formulation of *Staphylococcus aureus* protein A might be of significant benefit for patients with immune

[*]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 512,900,000 (Eurostat 2015).

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thrombocytopenia. Early studies show that the medicine could be useful in patients whose condition has returned or is not responding to treatment. 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?

The medicine is believed to modulate the activity of the body's immune cells, particularly their activity against 'opsonized' platelets. These are platelets that have already been marked out to be attacked by the immune cells. By reducing the activity of immune cells against opsonized platelets, the medicine reduces the clearance of platelets from the blood, thereby raising their blood levels and reducing the risk of bleeding.

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 immune thrombocytopenia were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for immune thrombocytopenia. Orphan designation of the medicine has been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 September 2015 recommending the granting of this designation.

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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

For details of the current sponsor of the orphan designation please refer to the information on the main web page of this Public Summary of Opinion.

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	A highly purified formulation of *Staphylococcus aureus* protein A	Treatment of immune thrombocytopenia
Bulgarian	Високо пречистен протеин А получен от *Staphylococcus auerus*	Лечение на имунна тромбоцитопения
Croatian	Visoko pročišćeni oblik proteina A iz *Staphylococcus* auerusa	Lječenje imune trombocitopenije
Czech	Vysoce čištěná formulace Stafylokokoku *auerus* protein A	Léčba imunitní trombocytopenie
Danish	Et højt oprenset formulering af *Staphylocuccus aureus* protein A	Behandling af immun trombocytopeni
Dutch	Een hoog gepurifiëerde formulering van *Staphylococcus auerus* proteïne A	Behandeling van immune trombocytopenie
Estonian	Kõrgpuhastatud formuleeritud Stafülokoki *auerus* A-valk	Immuungeneesiga trombotsütopeenia ravi
Finnish	Stafylokokki aureuksen proteiini A:n erittäin puhdistettu valmistemuoto	Immuunitrombosytopenian hoito
French	Une formulation hautement purifiée de Protéine A de *Staphylococcus aureus*	Traitement de la thrombopénie immunitaire
German	Eine hoch-aufgereinigte Formulierung von Staphylokokken *auerus* protein A	Behandlung von Immunthrombozytopenie
Greek	Υψηλής καθαρότητας σκεύασμα της πρωτεΐνης Α του χρυσίζοντος Σταφυλόκοκκου	Θεραπεία της ανοσολογικής θρομβοκυτταροπενίας
Hungarian	Nagy tisztaságú *Staphylococcus aureus* „A” fehérjekészítmény	Immun trombocitopénia kezelése
Italian	Formulazione altamente purificata della proteina A di *Staphylococcus aureus*	Trattamento della trombocitopenia immune
Latvian	Augstas tīrības pakāpes *Staphylococcus aureus* A proteīna preparāts	Imūnās trombocitopēnijas ārstēšana
Lithuanian	Labai išgrynintas *Staphylococcus aureus* A baltymas	Imuninės trombocitopenijos gydymas
Maltese	Formulazzjoni purifikata ħafna ta’ proteina A ta’ *Staphylococcus aureus*	Kura tat-trombożitopenja immuna
Polish	Wysoce oczyszczony preparatBiałka A ze *Staphylococcus aureus*	Leczenie małopłytkowości immunologicznej
Portuguese	Formulação altamente purificada de Proteína A de *Staphylococcus aureus*	Tratamento de trombocitopenia imune
Romanian	O formulă înalt purificată a proteinei A a *Staphylococcus aureus*	Tratamentul trombocitopeniei imune
Slovak	Vysoko čistená Formulácia *Staphylococcus aureus* proteínu A	Liečba imunitnej trombocytopénie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Visoko prečiščena formulacija proteina A Staphyllococcus aureusa	Zdravljenje imunske trombocitopenije
Spanish	Proteína A de *Staphylococcus aureus* altamente purificada	Tratamiento de la trombocitopenia inmune
Swedish	En höggradigt renad Formulering av *Staphylococcus aureus* protein A	Behandling av immuntrombocytopeni
Norwegian	Et høyt renset Formulering av Stafylokokk *aureus* protein A	Behandling av immun trombocytopeni
Icelandic	Háhreinsuð formúlering af *Staphylococcus aureus* próteín A	Meðferð ónæmistegdrii blóðflagnafæð