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EMA/COMP/553057/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene for the treatment of Wiskott-Aldrich syndrome

On 7 October 2013, orphan designation (EU/3/13/1196) was granted by the European Commission to Généthon, France, for autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene for the treatment of Wiskott-Aldrich syndrome.

What is Wiskott-Aldrich syndrome?

Wiskott-Aldrich syndrome is an inherited disease, seen almost exclusively in males, that affects blood cells and cells of the immune system (the body's natural defences). It is caused by abnormalities in the gene that produces the Wiskott-Aldrich syndrome (WAS) protein. Because patients with the condition lack the WAS protein, their immune cells and blood cells do not develop and function normally.

Patients with Wiskott-Aldrich syndrome have problems with bruising and bleeding easily because they have too few normal platelets (components that help the blood to clot), frequent infections because they have too few normal immune cells and eczema (itchy, red rash). In addition, there is a higher risk of developing some types of cancer, such as lymphoma.

Wiskott-Aldrich syndrome is life-threatening and long-term debilitating due to recurrent infections that can lead to sepsis (when bacteria and their toxins circulate in the blood and start damaging the organs), bleeding episodes and cancer.

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

At the time of designation, Wiskott-Aldrich syndrome affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 500 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).

[*]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 512,200,000 (Eurostat 2013).

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What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for Wiskott-Aldrich syndrome. Patients were managed with platelet transfusion to prevent bleeding and with immunoglobulin transfusion and antibiotics to prevent infections. A minority of patients was able to receive a bone marrow transplant.

How is this medicine expected to work?

This medicine is made up of immature bone marrow cells (called CD34+ cells) that are taken from the patient. These cells are able to develop into different types of blood and immune cells. To make this medicine, the cells are modified by a virus that contains the gene for the WAS protein, so that this gene is carried into the cells. When these modified cells are transplanted back into the patient, they are expected to populate the bone marrow and produce healthy blood and immune cells producing the WAS protein, which is lacking in patients with Wiskott-Aldrich syndrome.

The type of virus used in this medicine ('lentivirus') is modified in order not to cause disease in humans.

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 Wiskott-Aldrich syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for Wiskott-Aldrich syndrome 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 4 September 2013 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

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	Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene	Treatment of Wiskott-Aldrich syndrome
Bulgarian	Автоложни CD34+ клетки, трансдуцирани с лентивирусен вектор, съдържащ човешкия ген за синдрома на Wiskott-Aldrich	Лечение на Синдром на Wiskott-Aldrich
Czech	Autologní CD34 + bunky transdukované lentivirovým vektor obsahujícího lidský gen Wiskott-Aldrich SyndromeWiskott	Léčba Wiskott-Aldrichtova syndromu
Croatian	Autologne CD34+ stanice transducirane lentivirusnim vektorom koji sadrži ljudski gen Wiskott-Aldrichovog sindroma	Liječenje Wiskott-Aldrichovog sindroma
Danish	Autologe CD34 +-celler transduceret med en lentiviral vektor indeholdende det humane Wiskott-Aldrich Syndrome -gen	Behandling af Wiskott-Aldrich-syndrom
Dutch	Autologe CD34 + cellen getransduceerd met een lentivirale vector die het menselijk Wiskott-Aldrich Syndrome -gen bevat	Behandeling van het Wiskott-Aldricht-syndroom
Estonian	Autoloogsed CD34+ rakud, millesse on viidud lentiviraalne vektor, mis sisaldab inimese Wiskott-Aldrich Syndrome geeniWiskott	Wiskott-Aldrichti sündroomi ravi
Finnish	Autologisia CD34+ -soluja, jotka on transdusoitu lentivirusvektorilla, joka sisältää ihmisen Wiskott-Aldrich oireyhtymägeeninWiskott	Wiskott-Aldrichin oireyhtymän hoito
French	Cellules CD34 + autologues transduites avec un vecteur lentiviral contenant le gène humain du Syndrome Wiskott-Aldrich	Traitement du syndrôme de Wiskott-Aldrich
German	Autologe CD34 +-Zellen transduziert mit einem lentiviralen Vektor, der das humane Wiskott-Aldrich Syndrome Gen enthält	Behandlung des Wiskott-Aldrich-syndrom
Greek	Αυτόλογα κύτταρα CD34 διαμολυσμένα με λεντι-φορέα που περιέχει το ανθρώπινο γονίδιο του Wiskott-Aldrich συνδρόμου	Θεραπεία του συνδρόμου Wiskott-Aldrich
Hungarian	Humán Wiskott-Aldrich szindróma gént tartalmazó lentivírussal transzformált autolog CD34+ sejtek	Wiscott-Aldrich szindróma kezelése
Italian	Cellule autologhe CD34 + trasdotte con un vettore lentivirale contenente il gene umano della Wiskott-Aldrich SyndromeWiskott	Trattamento della sindrome di Wiskott-Aldrich

[1] At the time of designation

Language	Active ingredient	Indication
Latvian	Autologas CD34+ šūnas pārveidotas ar lentivīrusa vektoru, kas satur cilvēka Viskota-Oldriča sindroma gēnuWiskott	Viskota-Oldriča sindroma ārstēšana
Lithuanian	Autologinės CD34 + ląstelės pakeistos lentivirusiniu vektoriumi, koduojančiu žmogaus *Wiskott-Aldrich* sindromo geną	Viskoto-Aldricho (Wiskott-Aldrich) sindromo gydymas
Maltese	Ċelluli CD34+ awtologi trasformati b'vettur lentivirali li fih il-ġene uman tas-sindrome Wiskott-AldrichWiskott	Kura tas-sindrome ta' Wiskott-Aldrich
Polish	Autologiczne komórki CD34 + transdukowane wektorem lentiwirusowym zawierającym ludzki gen zespołu Wiskotta-Aldricha	Leczenie zespołu Wiskott-Aldricha
Portuguese	Células CD34 + autólogas transduzidas com um vector lentiviral contendo o gene humano do síndrome de Wiskott-AldrichWiskott	Tratamento do síndrome de Wiskott-Aldrich
Romanian	Celule CD34 + autologe transduse cu un vector lentiviral care conţine gena umană a Sindromului Wiskott-AldrichWiskott	Tratamentul sindromului Wiskott-Aldrich
Slovak	Autológne CD34 + bunky transdukované lentivírusovým vektorom obsahujúcim ľudský gén pre WiskottWiskottov-Aldrichov syndróm	Liečba Wiskottovho-Aldrichovho syndrómu
Slovenian	Avtologne celice CD34 + transducirane z lentivirusnim vektorjem, ki vsebuje humani gen za Wiskott-Aldrichov sindromWiskott	Zdravljenje Wiskott-Aldrich sindroma
Spanish	Células CD34 + autologas transducidas con un vector lentiviral que contiene el gen humano del síndrome de Wiskott-Aldrich	Tratamiento del síndrome de Wiskott-Aldrich
Swedish	Autologa CD34+ celler transducerade med en lentivirusvektor innehållande den humana Wiskott-Aldrich Syndrom–genen	Behandling av Wiskott-Aldrichs syndrom
Norwegian	Autologe CD34 +-celler transdusert med en lentiviral vektor inneholdende det humane Wiskott-Aldrich Syndrom genetWiskott	Behandling av Wiskott-Aldrichs syndrom
Icelandic	Samgena CD34 + frumur transduced með lentiveiru ferjusem inniheldur manna Wiskott-Aldrich heilkennis genWiskott	Meðferð við Wiskott-Aldrich heilkenni