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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene for the treatment of Wiskott-Aldrich syndrome

First publication	2 July 2012
Rev.1: sponsor's change of address	20 November 2013
Rev.2: transfer of sponsor	4 March 2015

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 6 June 2012, orphan designation (EU/3/12/998) was granted by the European Commission to Fondazione Telethon, Italy, for autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene for the treatment of Wiskott-Aldrich syndrome.

The sponsorship was transferred to GlaxoSmithKline Trading Services Limited, Ireland, in December 2014.

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 protein (WASP), found in blood cells and certain immune cells. Because patients with the condition lack the WASP 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.

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Wiskott-Aldrich syndrome is life-threatening and long-term debilitating due to recurrent infections that could lead to sepsis (when bacteria and their toxins circulate in the blood and starts 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)

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for Wiskott-Aldrich syndrome. A minority of patients was able to receive a bone marrow transplant (those at a very young age and having a compatible donor).

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 WASP 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 that produce the WASP protein, which is lacking in patients with Wiskott Aldrich syndrome, and thereby help to relieve the symptoms of the disease.

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, 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. Orphan designation of the medicine had been granted in the United States of America for the treatment of Wiskott-Aldrich syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 April 2012 recommending the granting of this designation.

[*]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.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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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http://ie.gsk.com/ie/contact-us

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 transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene	Treatment of Wiskott-Aldrich syndrome
Bulgarian	Автоложни CD34+ клетки, трансфектирани с лентивирусен вектор, съдържащ човешки ген на протеина на синдрома на Wiskott-Aldrich	Лечение на Синдром на Wiskott-Aldrich
Croatian	Autologne CD34+ stanice transficirane lentivirusnim vektorom koji sadrži gen za protein Wiskott-Aldrichovog sindroma	Liječenje Wiskott-Aldrichovog sindroma
Czech	Autologní CD34+ buňky s lentivirálním vektorem obsahujícím lidský gen proteinu Wiskott-Aldrich syndromu	Léčba Wiskott-Aldrichtova syndromu
Danish	Autologe CD34+ celler transfekterede med en lentiviral vector som indeholder det humane Wiskott Aldrich syndrom protein gen	Behandling af Wiskott-Aldrich-syndrom
Dutch	Autologe CD34+ cellen getransfecteerd met een lentivirale vector met het humaan Wiskott Aldrich syndroom proteïne gen	Behandeling van het Wiskott-Aldricht-syndroom
Estonian	Autoloogsed CD34+ rakud transfekteeritud lentiviiruslik vektoriga, mis sisaldab Wiskott Aldrichi sündroomi valgu geeni	Wiskott-Aldrichti sündroomi ravi
Finnish	Autologisia CD34+ soluja, jotka ovat transfektoituja lentivirusvektorilla, joka sisältää Wiskott Aldrich syndrom proteinhumaanigeenin	Wiskott-Aldrichtin oireyhtymän hoito
French	Cellules autologues CD34+ transfectées avec un vecteur lentiviral contenant le gène de la protéine du syndrôme de Wiskott-Aldrich	Traitement du syndrôme de Wiskott-Aldrich
German	Autologe CD34+ Zellen, transfiziert mit lentiviralen Vektoren, die das Gen des humanen Wiskott Aldrich Syndrom Proteins enthalten	Behandlung des Wiskott-Aldrich-syndrom
Greek	Αυτόλογα κύτταρα CD34+ επιμολυσμένα με φορέα λεντι-ιού που περιέχει το ανθρώπινο γονίδιο της πρωτεΐνης του συνδρόμου Wiskott Aldrich	Θεραπεία του συνδρόμου Wiskott-Aldrich
Hungarian	Emberi Wiscott-Aldricht szindróma fehérje génjét horhozó autológ CD34+ sejtekkel egyesített lenti vírus vektor	Wiscott-Aldrich szindróma kezelése
Italian	Cellule CD34+ autologhe transfettate con vettore lentivirale contenente il gene umano della proteina della sindrome di Wiskott Aldrich	Trattamento della sindrome di Wiskott-Aldrich

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Latvian	Autologas CD34+ šunas ar ievadītu cilvēka Viskota-Oldriča sindroma proteīna gēnu saturošu lentiviralu vektoru	Viskota-Oldriča sindroma ārstēšana
Lithuanian	Autologinės CD34+ lastelės su įvestu lentiviruso vektoriumi, turinčiu Viskoto-Aldricho (Wiskott-Aldrich) sindromo gena koduojanti baltyma	Viskoto-Aldricho (Wiskott-Aldrich) sindromo gydymas
Maltese	Ċelluli CD34+ awtologi transfettati b'vettur lentivirali li fih il-ġene tal-proteina tas-sindrome ta' Wiskott Aldrich	Kura tas-sindrome ta' Wiskott-Aldrich
Polish	Autologiczne komórki CD34+ transfekowane lentiwirusowym wektorem zawierającym gen białka zespołu Wiskott-Aldricha	Leczenie zespołu Wiskott-Aldricha
Portuguese	Células autólogas CD34+ transfectadas com um vector lentiviral que contém o gene humano da proteína do síndrome de Wiskott-Aldrich	Tratamento do síndrome de Wiskott-Aldrich
Romanian	Celule CD34+ autologe prelucrate prin transfecţie cu vector lentiviral care conţine gena proteinei sindromului Wiskott Aldrich	Tratamentul sindromului Wiskott-Aldrich
Slovak	Autológne CD34+ bunky transfektované lentivírusovým vektorom obsahujúcim gén pre proteín Wiskottovho-Aldrichovho syndrome	Liečba Wiskottovho-Aldrichovho syndrómu
Slovenian	Avtologne CD34+ celice transficirane z lentivirusnim vektorjem ki vsebuje humani gen sindroma Wiscott-Aldrich	Zdravljenje Wiskott-Aldrich sindroma
Spanish	Células CD34+ autólogas transfectadas con un vector lentivírico que contiene el gen humano de la proteína del síndrome de Wiskott-Aldrich	Tratamiento del síndrome de Wiskott-Aldrich
Swedish	Autologa CD43+ celler transfekterade med lentivirusvektor innehållande genen för Wiskott-Aldrich syndrom protein	Behandling av Wiskott-Aldrichs syndrom
Norwegian	Autologe CD34+ celler transfektert med lentiviral vektor som inneholder genet for Wiskott-Aldrichs syndrom protein	Behandling av Wiskott-Aldrichs syndrom
Icelandic	Samgena CD34+ frumutransfected með lentiveiru ferju sem innheldur Wiskott Aldrich heilkennis protein gen.	Meðferð við Wiskott-Aldrich heilkenni