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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lentiviral vector containing the human Wiskott Aldrich syndrome protein gene for the treatment of Wiskott Aldrich syndrome

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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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in May 2014 on request of the Sponsor.

On 24 January 2006, orphan designation (EU/3/05/345) was granted by the European Commission to Généthon, France, for lentiviral vector containing the human Wiskott Aldrich syndrome protein gene for the treatment of Wiskott Aldrich syndrome.

What is Wiskott Aldrich syndrome?

A chromosome is an organised structure of DNA (the genetic material) and protein that is found in cells of the body. In humans, the so-called X and Y-chromosomes determine the sex, but carry also other genetic information. Wiskott Aldrich syndrome is caused by an abnormality of a gene located on the X chromosome. This gene is responsible for the production of a protein, Wiskott Aldrich protein, which is part of the structure responsible for the cellular "scaffolding" enabling the shape and movement of the cells (cytoskeleton). The cytoskeleton is also fundamental for the cell to be able to capture substances or organisms (endocytosis) or for actions linked to the body's defense system. This means that patients suffering from Wiskott Aldrich syndrome have different symptoms, depending on the cells affected, such as decrease in the number of platelets and increased bleeding, eczema, repeated infections, or increased risk for development of autoimmune diseases and tumours.

Wiskott Aldrich syndrome is chronically debilitating and life-threatening.



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?

No satisfactory methods exist that were authorised at the time of application. For a minority of patients (those at a very young age and having a compatible donor) bone marrow transplant is a therapeutic option. Medicines generally available for the complications of the disease are used in these situations.

How is this medicine expected to work?

Lentiviral vector containing the human Wiskott Aldrich syndrome protein gene is a medicinal product that uses a virus to carry the normal gene necessary for the production of the Wiskott Aldrich protein. A virus is a small organism capable of introducing genetic material into the cells. The type of virus (lentivirus) used in this medicinal product is modified in order to avoid causing any disease in humans. By replacing the abnormal gene, this product is expected to produce the Wiskott Aldrich protein and thus improve the symptoms of the patients.

What is the stage of development of this medicine?

The effects of lentiviral vector containing the human Wiskott Aldrich syndrome protein gene were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with Wiskott Aldrich syndrome condition were initiated.

Lentiviral vector containing the human Wiskott Aldrich syndrome protein gene was not authorised anywhere worldwide for Wiskott Aldrich syndrome or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 December 2005 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.

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Lentiviral vector containing the human Wiskott Aldrich syndrome protein gene	Treatment of Wiskott-Aldrich syndrome
Czech	Lentivirový vektor obsahující lidský gen proteinu Wiskott-Aldrichtova syndromu	Léčba Wiskott-Aldrichtova syndromu
Danish	Lentiviral vektor indeholdende det humane Wiskott Aldrich syndrom protein gen	Behandling af Wiskott-Aldrich-syndrom
Dutch	Lentivirale vector met het humaan Wiskott Aldrich syndroom proteïne gen	Behandeling van het Wiskott-Aldrich-syndroom
Estonian	Lentiviiruslik vektor, mis sisaldab inimese Wiskott Aldrichi sündroomi valgu geeni	Wiskott-Aldrichi sündroomi ravi
Finnish	Lentivirusvektori, joka sisältää Wiskott Aldrich syndrom proteinhumaanigeenin	Wiskott-Aldrichtin oireyhtymän hoito
French	Vecteur lentiviral contenant le gène humain de la protéine du syndrome de Wiskott-Aldrich	Traitement du syndrome de Wiskott-Aldrich
German	Lentivirale Vektoren, die das Gen des Proteins des humanen Wiskott Aldrich Syndroms enthalten	Behandlung des Wiskott-Aldrich-syndrom
Greek	Εφηλιδικός φορέας ιού περιέχων το ανθρώπινο γονίδιο της πρωτεΐνης του συνδρόμου Wiskott Aldrich	Θεραπεία του συνδρόμου Wiskott-Aldrich
Hungarian	Emberi Wiscott-Aldrich szindróma fehérje génjét hordozó Lenti virus vector	Wiscott-Aldrich szindróma kezelése
Italian	Vettore derivato da lentivirus contenente il gene umano per la proteina della sindrome di Wiskott Aldrich	Trattamento della sindrome di Wiskott-Aldrich
Latvian	Lentivīrusu vektors, kas satur cilvēka Viskota-Oldriča sindroma proteīna gēnu	Viskota-Oldriča sindroma ārstēšana
Lithuanian	Lentiviruso vektorius su žmogaus Wiskott-Aldrich sindromo geno baltymu	Wiskott-Aldrich (Viskoto-Oldričo) sindromo gydymas
Polish	Wektor lentiwirusa zawierający gen ludzkiego białka zespołu Wiskott-Aldricha	Leczenie zespołu Wiskott-Aldricha
Portuguese	Vector lentiviral contendo o gene humano da proteína do síndrome Wiskott-Aldrich	Tratamento do síndrome Wiskott-Aldrich
Slovak	Lentivírusový vektor obsahujúci ľudský gén pre proteín Wiskott-Aldrichovho WASP syndrómu	Liečba Wiskott-Aldrichovho syndrómu
Slovenian	Lentivirusni vektor za humani gen sindroma Wiscott-Aldrich	Zdravljenje Wiskott-Aldrich sindroma

¹ At the time of designation

Language	Active Ingredient	Indication
Spanish	Vector lentiviral que contiene el gen humano de la proteína del síndrome de Wiskott-Aldrich	Tratamiento del síndrome de Wiskott-Aldrich
Swedish	Lentivirusvektor innehållande den humana Wiskott-Aldrich syndrom protein -genen	Behandling av Wiskott-Aldrichs syndrom
Norwegian	Lentivirusvektor som inneholder humant Wiskott Aldrichs syndrom protein -gen	Behandling av Wiskott-Aldrichs syndrom
Icelandic	Leentiveiru ferja sem inniheldur manna Wiskott Aldrich heilkennis prótein genið	Meðferð við Wiskott-Aldrich heilkenni