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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adeno-associated viral vector containing the human gamma sarcoglycan gene for the treatment of gamma sarcoglycanopathy

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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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in May 2014 on request of the Sponsor.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 21 October 2004, orphan designation (EU/3/04/233) was granted by the European Commission to Généthon, France, for adeno-associated viral vector containing the human gamma sarcoglycan gene for the treatment of gamma sarcoglycanopathy.

What is gamma sarcoglycanopathy?

Gamma sarcoglycanopathy is an inherited condition and usually appears in childhood. The abnormal gene is located on a portion of chromosome number 13, and the disease occurs if both parents pass an abnormal version of this gene onto their offspring (this type of disease transmission is called "autosomal recessive" transmission). Patients suffering from this condition are not able to produce a protein called gamma sarcoglycan in the muscle cells. This protein is important for normal functioning of the muscle cells. Gamma sarcoglycanopathy is characterised by progressive weakness of the muscles. Gamma sarcoglycanopathy is chronically debilitating and life-threatening.

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

At the time of designation, gamma sarcoglycanopathy affected approximately 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of 1,300 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 submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition. Treatment of patients with gamma sarcoglycanopathy primarily involved physiotherapy as supportive treatment, but also surgery of the tendon and spine fusion.

How is this medicine expected to work?

Adeno-associated viral vector containing the human gamma sarcoglycan gene is a medicinal product which uses a virus to carry the gene necessary for the production of the gamma sarcoglycan protein. A virus is a small organism capable of introducing genetic material in cells. The type of virus (adeno-associated virus) used in this medicinal product is modified in order to avoid causing any disease in humans.

What is the stage of development of this medicine?

The evaluation of the effects of adeno-associated viral vector containing the human gamma sarcoglycan gene in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with gamma sarcoglycanopathy were initiated.

Adeno-associated viral vector containing the human gamma sarcoglycan gene was not marketed anywhere worldwide for gamma sarcoglycanopathy 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 9 September 2004 recommending the granting of this designation.

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*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 464,200,000 (Eurostat 2004).

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://www.genethon.fr/en/contacts-en/

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	Adeno-associated viral vector containing the human gamma-sarcoglycan gene	Treatment of gamma-sarcoglycanopathy
Czech	Adenovirový vektor s lidským genem pro gamma-sarkoglykan	Léčba gamma-sarkoglykanopatie
Danish	Adenoassocieret viral vektor indeholdende det humane gamma-sarcoglycan-gen	Behandling af gamma-sarcoglycanopati
Dutch	Adenovirale vector die het humaan gamma-sarcoglycaan-gen bevat.	Behandeling van gamma-sarcoglycanopathie
Estonian	Adenoga assotsieeruv viirusvektor, mis sisaldab inimese gamma-sarkoglükaani geeni	Gamma-sarkoglükanopaatia ravi
Finnish	Adenovirusvektori, joka sisältää gamma-sarkoglukaani –humaanigeenin	Gamma-sarkoglukanopatian hoito
French	Vecteur viral adéno-associé contenant le gène humain du gamma-sarcoglycane	Traitement de la gamma-sarcoglycanopathie
German	Adeno-assoziierter viraler Vektor, der das human gamma-sarkoglykan-gen enthält	Behandlung von gamma-Sarkoglykanopathie
Greek	Αδενοσύνδετος φορέας ιού περιέχων το γονίδιο του ανθρώπινου γάμμα-σαρκοπολυσακχαριδίου	Θεραπεία της γάμμα-σαρκοπολυσακχαριδοπάθειας
Hungarian	Emberi gamma-szarkoglikán gént hordozó adenovirus vektor	Gamma-sarcoglycanopathia kezelése
Italian	Vettore derivato da virus adeno-associati contenente il gene del gamma-sarcoglicano umano	Trattamento della gamma-sarcoglicanopatia
Latvian	Adeno-saistīts vīrusu vektors, kas satur cilvēka gamma-sarkoglikāna gēnu	Gamma-sarkoglikanopātijas ārstēšana
Lithuanian	Adeno-susijusio viruso vektorius su žmogaus gama-sarkoglikano genu	Gama sarkoglikanopatijos gydymas
Maltese	Adeno-associated viral vector containing the human gamma-sarcoglycan gene	Treatment of gamma-sarcoglycanopathy
Polish	Wektor rekombinowanego wirusa sprzężonego z adenowirusem zawierający ludzki gen gamma-sarkoglikanu	Leczenie gamma-sarkoglikanopatii
Portuguese	Vector viral adeno-associado contendo o gene humano gama-sarcoglican	Tratamento da gama-sarcoglicanopatia
Slovak	Adeno-asociovaný vírusový vektor obsahujúci ľudský gén gama-sarkoglykan	Liečba gama-sarkoglykanopatie

[1] At the time of designation

Language	Active Ingredient	Indication
Slovenian	Adenovirusom podobni virusni vektor s humanim genom za gama sarkoglikan	Zdravljenje δ-sarkoglikanopatije
Spanish	Vector viral adenoasociado que contiene el gen humano del gamma-sarcoglicano	Tratamiento de la gamma-sarcoglicanopatía
Swedish	Adenoassocierad virusvektor som innehåller den humana gamma-sarcoglycangenen	Behandling av gamma-sarcoglycanopati
Norwegian	Adenoassosiert virusvektor som inneholder humant gamma-sarkoglykangen	Behandling av gamma-sarkoglykanopati
Icelandic	Adenóveirutengd ferja sem inniheldur gamma-sarkóglýkan gen úr mönnum	Meðferð við gamma-sarkóglýkankvilla