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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adenovirus associated viral vector serotype 4 containing the human RPE65 gene for treatment of Leber's congenital amaurosis

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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.	

On 22 October 2007, orphan designation (EU/3/07/484) was granted by the European Commission to Centre Hospitalier Universitaire de Nantes, France, for adenovirus associated viral vector serotype 4 containing the human RPE65 gene for treatment of Leber's congenital amaurosis.

The sponsorship was transferred to HORAMA SAS, France, in May 2015.

What is Leber's congenital amaurosis?

Leber's congenital amaurosis is an inherited disease characterised by progressive loss of sight. Approximately 6% of patients affected by Leber's congenital amaurosis have a defective RPE65 gene, and thus do not produce the RPE65 protein in the cells in the eye. Leber's congenital amaurosis is chronically debilitating due to progressive loss of vision.

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

At the time of designation, Leber's congenital amaurosis affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 50,000 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).

*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 500,300,000 (Eurostat 2007).



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. Patients with Leber's congenital amaurosis usually receive genetic counselling and general support such as information and regular medical follow up.

How is this medicine expected to work?

Adenovirus associated viral vector serotype 4 containing the human RPE65 gene is a medicinal product that uses a virus to carry the gene necessary for the production of the protein RPE65. A virus is a small organism capable of introducing genetic material into cells. The type of virus (adeno-associated virus) used in this medicinal product is modified to avoid causing disease in humans.

What is the stage of development of this medicine?

The evaluation of the effects of adenovirus associated viral vector serotype 4 containing the human RPE65 gene in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with Leber's congenital amaurosis had been initiated.

Adenovirus associated viral vector serotype 4 containing the human RPE65 gene was not authorised anywhere in the world for Leber's congenital amaurosis 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 12 September 2007 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.

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:

HORAMA SAS
9 rue de l'Eperon
75006 Paris
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Tel. +33 6 22 73 26 34
<http://www.horama.fr/contact/>

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	Adenovirus associated viral vector serotype 4 containing the human RPE65 gene	Treatment of Leber's congenital amaurosis
Bulgarian	Адено-асоцииран вирусен вектор серотип 4, съдържащ човешкия ген RPE65	Лечение на вродена амавроза на Лебер
Croatian	Adeno-povezani virusni vektor serotipa 4 koji sadrži ljudski gen RPE65	Liječenje Leberove kongenitalne amauroze
Czech	Sérotyp 4 virového vektoru spojeného s adenovirem, obsahující lidský gen RPE65	Léčba Leberovy vrozené slepoty
Danish	Adenovirus associeret viral vektor serotype 4 indeholdende det humane gen RPE65	Behandling af Lebers kongenitte amaurose
Dutch	Adenovirus geassocieerde virale vector, serotype 4, welke het humane gen RPE65 bevat	Behandeling van amaurosis congenita van Leber
Estonian	Adenoviirusega ühendatud viirusvektori serotüüp 4, mis sisaldab inimese RPE65 geeni	Leberi tüüpi pärilik amauroos (nägemisnärvi kõhetusest tingitud pimedus) ravii
Finnish	Adenovirukseen liittyvä, serotyyppiä 4 oleva virustartunnanvälittäjä, joka sisältää ihmisen RPE65 geenin	Leberin synnyynnäisen amauroosin (sokeus) hoito
French	Vecteur viral adénovirus-associé de sérotype 4 contenant le gène humain RPE65	Traitement de l'amaurose congénitale de Leber
German	Das humane RPE65 Gen beinhaltender, adenovirus-assoziiertes viraler Vektor Serotyp 4	Behandlung der Leberschen Kongenitalen Amaurose
Greek	Ιογενής φορέας συνδεδεμένος με αδενοϊό, ορότυπου 4 που περιέχει το ανθρώπινο γονίδιο RPE65	Θεραπεία τής συγγενούς αμαύρωσης του Leber
Hungarian	Humán RPE65 gént tartalmazó 4-es szerotípusú adenovírus vektor	Leber-féle hereditaer opticus atrophia kezelése
Italian	Vettore virale adenovirus-associato del serotipo 4 contenente il gene umano RPE65	Trattamento dell'amaurosi congenita di Leber
Latvian	Adenovīrusa Saistītā Virālā vektora 4. serotips, kas satur cilvēka RPE65 gēnu	Iedzimta Lēbera akluma ārstēšana
Lithuanian	Adeno - asocijuotas virusinio vektoriaus 4 serotipas, pernešantis žmogaus RPE65 geną	Įgimtos Lėberio amaurozės gydymas
Maltese	Vektor virali assoċjat ma' l-adenovirus tas-serotip 4 li għandu l-ġene uman RPE65	Kura ta' l-amawrozi kongenitali ta' Leber
Polish	serotyp 4 wektora wirusowego związanego z adenowirusem, zawierający ludzki gen RPE65	Leczenie wrodzonej ślepoty Lebera

¹ At the time of designation

Language	Active Ingredient	Indication
Portuguese	Vector viral associado ao adenovirus de serotipo 4 contendo o gene humano RPE65	Tratamento do Amaurose Congénita de Leber
Romanian	Vector viral asociat adenovirusului de serotip 4 conținând gena umană RPE65	Tratamentul amaurozei congenitale Leber
Slovak	Adenovírusový pridružený vírusový vektorový sérotyp 4 obsahujúci ľudský gén RPE65	Liečba Leberovej vrodenej amaurozy
Slovenian	Adenovirusom sorodni virusni vektor serotipa 4, ki vsebuje človeški gen RPE65	Zdravljenje Leberjeve vrojene amauroze
Spanish	Vector vírico asociado al adenovirus del serotipo 4 que contiene el gen humano RPE65	Tratamiento de la amaurosis congénita de Leber
Swedish	Adenoassocierat virus, viral vektor serotyp 4, innehållande den mänskliga genen RPE65	Behandling av Lebers kongenitala amauros
Norwegian	Adenoassosiert virusvektor serotype 4 som inneholder det humane genet RPE65	Behandling av Lebers kongenitte amaurose
Icelandic	Adenóveirutengd (af flokki 4) veirufurja sem inniheldur RPE65 gen úr manni	Meðferð á Leber meðfæddri blindu