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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lentiviral vector containing the human *MYO7A* gene for the treatment of retinitis pigmentosa in Usher syndrome 1B

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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 23 March 2010, orphan designation (EU/3/10/727) was granted by the European Commission to Oxford BioMedica (UK) Ltd, United Kingdom, for lentiviral vector containing the human *MYO7A* gene for the treatment of retinitis pigmentosa in Usher syndrome 1B.

The sponsorship was transferred to Sanofi-Aventis Recherche & Développement, France, in June 2014.

What is retinitis pigmentosa in Usher syndrome 1B?

Retinitis pigmentosa is group of hereditary diseases of the eye that lead to progressive loss of sight. In patients with retinitis pigmentosa, cells in the retina (the light-sensitive surface at the back of the eye) become damaged and eventually die. Retinitis pigmentosa is seen in a variety of diseases, including a genetic condition known as Usher syndrome. This condition also causes other problems, including deafness.

Retinitis pigmentosa in Usher's syndrome is a long-term debilitating disease because it causes the patient's sight to get worse, eventually leading to blindness.

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

At the time of designation, retinitis pigmentosa in Usher syndrome 1B affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 people*, and

*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 506,300,000 (Eurostat 2010).

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is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 methods were authorised in the EU for treating retinitis pigmentosa in Usher syndrome 1B. Patients with the condition were given sunglasses to slow down the damage to the retina, genetic counselling (discussion of the risks of passing the condition on to children) and general support.

How is this medicine expected to work?

Retinitis pigmentosa in Usher syndrome 1B is caused by abnormalities in a gene called *MYO7A*. This gene is involved in the normal development of the retina. Lentiviral vector containing the human *MYO7A* gene is an advanced therapy medicine that belongs to the group called 'gene therapy products'. These are medicines that work by delivering genes into the body. The medicine is made up of a virus that contains normal copies of the human *MYO7A* gene. When the medicine is injected into the eye, the virus is expected to carry the normal *MYO7A* gene into the cells of the retina, which will replace the abnormal gene in Usher syndrome 1B. This is expected to reduce or reverse the damage to retina cells, slowing down or preventing the loss of vision.

The type of virus used in this medicine (lentivirus) is modified so that it does not cause disease in humans.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of lentiviral vector containing the human *MYO7A* gene in experimental models was ongoing.

At the time of submission, no clinical trials with this medicine had been started in patients with retinitis pigmentosa in Usher syndrome 1B.

At the time of submission, lentiviral vector containing the human *MYO7A* gene was not authorised anywhere in the EU for retinitis pigmentosa in Usher syndrome 1B 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 6 January 2010 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	Lentiviral vector containing the human *MYO7A* gene	Treatment of retinitis pigmentosa in Usher syndrome 1B
Bulgarian	Лентивирусен вектор, съдържащ човешки *MYO7A* ген	Лечение на пигментен ретинит при синдром на Usher 1B
Croatian	Lentivirusni vektor koji sadrži ljudski gen *MYO7A*	Liječenje retinitisa pigmentoze u Usherovom sindromu 1B
Czech	Lentivirový vektor obsahující lidský gen *MYO7A*	Léčba pigmentosní retitinitidy u Usherova syndromu 1B
Danish	Lentiviral vektor indeholdende det humane *MYO7A* gen	Behandling af retinitis pigmentosa i Usher syndrom type I
Dutch	Lentivirale vector welke humaan *MYO7A* gen bevat	Behandeling van retinitis pigmentosa in Usher syndroom 1B
Estonian	Inimese *MYO7A* geeni sisaldav lentiviraalne vektor	Uhser 1B sündroomi pigmentoosse võrkkestapõletiku ravi
Finnish	Lentivirusvektori, jossa on ihmisen *MYO7A*-geeni–	Usherin oireyhtymään liittyvän verkkokalvon pigmenttisurkastuman hoito
French	Vecteur lentiviral contenant le gène humain *MYO7A*	Traitement de la rétinite pigmentaire du syndrome 1B de Usher
German	Lentiviraler Vektor, der das menschliche Gen *MYO7A* enthält	Behandlung einer Usher Syndrom 1B assoziierten Retinitis Pigmentosa
Greek	Όχημα lenti-ιού που φέρει το ανθρώπινο γονίδιο *MYO7A*	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας σε σύνδρομο Usher 1B
Hungarian	Humán *MYO7A* gént tartalmazó lentivirális vektor	Usher 1B szindrómában előforduló retinitis pigmentosa kezelése
Italian	Vettore lentivirale contenente il gene umano *MYO7A*	Trattamento della retinite pigmentosa nella sindrome di Usher di tipo 1B
Latvian	Lentavīrusa vektoru saturošs cilvēka *MYO7A* gēns	Retinitis pigmentosa ārstēšana pie Ušera (Usher) 1B sindroma
Lithuanian	Lentivirusinis vektorius, turintis žmogaus *MYO7A* geną	Pigmentinio retinito, sergant Usher'io sindromo 1B tipu, gydymas
Maltese	Vettur lentivirali li fih il-ġene *MYO7A* uman	Kura tar-retinite pigmentuża fis-sindrome 1B ta' Usher
Polish	Wektor lentiwirusowy zawierający ludzki gen *MYO7A*	Leczenie retinopatii barwnikowej w zespole Ushera typu 1B
Portuguese	Vector lentiviral contendo o gene *MYO7A* humano	Tratamento da retinite pigmentosa no sindrome 1B de Usher
Romanian	Vector lentiviral conţinând gena umană *MYO7A*	Tratamentul retinitei pigmentare din sindromul Usher 1B
Slovak	Lentivírusový vektor obsahujúci ľudský gén *MYO7A*	Liečba pigmentovej retinitídy pri Usherovom syndróme 1B
Slovenian	Lentivirusni vector s humanim genom *MYO7A*	Zdravljenje pigmentozne retinopatije pri Usherjevem sindromu 1B

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Vector lentivírico que contiene el gen humano *MYO7A*	Tratamiento de retinosis pigmentaria en el síndrome de Usher 1B
Swedish	Lentivirusvektor innehållande den humana *MYO7A* -genen	Behandling av retinitis pigmentosa med Ushers syndrom 1B
Norwegian	Lentiviral vektor som inneholder genet for human *MYO7A*	Behandling av retinitis pigmentosa ved Ushers syndrom 1B
Icelandic	Lentiveiru ferja sem inniheldur manna *MYO7A* genið	Meðferð á retinitis pigmentosa í Usher heilkenni 1B