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EMA/COMP/446355/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adeno-associated viral vector serotype 2.7m8 containing the *ChrimsonR-tdTomato* gene for the treatment of retinitis pigmentosa

On 14 July 2016, orphan designation (EU/3/16/1693) was granted by the European Commission to GenSight Biologics, France, for adeno-associated viral vector serotype 2.7m8 containing the *ChrimsonR-tdTomato* gene for the treatment of retinitis pigmentosa.

What is retinitis pigmentosa?

Retinitis pigmentosa is a 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 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 affected less than 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 190,000 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).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for treating retinitis pigmentosa. 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.

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

In patients with retinitis pigmentosa, the light-sensitive cells in the retina gradually die. The medicine consists of a virus that has been modified to contain copies of a gene that produces a light-sensitive pigment. When injected into the eye, the medicine is expected to carry this gene into nerve cells in the retina that are not normally sensitive to light. This would enable these cells to produce the pigment and become responsive to light, so helping to restore the action of the retina and reduce symptoms of the condition.

The virus used in this medicine (adeno-associated virus) does not 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 of the application for orphan designation, no clinical trials with this medicine in patients with retinitis pigmentosa had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for retinitis pigmentosa 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 16 June 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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 serotype 2.7m8 containing the *ChrimsonR-tdTomato* gene	Treatment of retinitis pigmentosa
Bulgarian	Адено-свързан вирусен вектор, серотип 2.7m8 съдържащ ген *ChrimsonR-tdTomato*	Лечение на пигментен ретинит
Croatian	Adeno-povezani virusni vektor serotipa 2.7m8 koji sadrži gen *the ChrimsonR-tdTomato*	Liječenje retinitisa pigmentoze
Czech	Adeno-asociovaný virus sérotypu 2.7m8 obsahující gen *ChrimsonR-tdTomato*	Léčba pigmentosní retinitidy
Danish	Adenovirus associeret viral vektor serotype 2.7m8 indeholdende *ChrimsonR-tdTomato* genet	Behandling af retinitis pigmentosa
Dutch	Adenovirus geassociëerde virale vector serotype 2.7m8, welke het *ChrimsonR-tdTomato* gen bevat	Behandeling van retinitis pigmentosa
Estonian	Adenoviirusega seotud viirusvektor serotüüp 2.7m8 , mis sisaldab *ChrimsonR-tdTomato* geeni	Pigmentoosse võrkkestapõletiku ravi
Finnish	Serotyypin 2.7m8 adenovirusvektori, jossa on *ChrimsonR-tdTomato*-geeni	Verkkokalvorappeuman hoito
French	Vecteur viral adéno-associé de type 2.7m8 contenant le gène *ChrimsonR-tdTomato*	Traitement de la rétinite pigmentaire
German	Adenovirus-assoziierter viraler Vektor Serotyp 2.7m8, der das *ChrimsonR-tdTomato* Gen enthält	Behandlung der Retinopathia Pigmentosa
Greek	Ιικός φορέας σχετιζόμενος με αδενοϊό ορότυπου 2.7m8 που περιέχει το γονίδιο *ChrimsonR-tdTomato*	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	*ChrimsonR-tdTomato* gént tartalmazó 2.7m8-as szerotípusú adeno-asszociált vírus vektor	Retinitis pigmentosa kezelése
Italian	Vettore virale adenovirus-associato del serotipo 2.7m8 contenente il gene *ChrimsonR-tdTomato*	Trattamento della retinite pigmentosa
Latvian	Adeno-saistīts 2.7m8 serotipa vīrusa vektors, kas satur *ChrimsonR-tdTomato* gēnu	*Retinitis pigmentosa* ārstēšana
Lithuanian	Su adenovirusu asocijuotas 2.7m8 serotipo virusinis vektorius, turintis *ChrimsonR-tdTomato* geną	Pigmentinio retinito gydymas
Maltese	Vettur virali mnissel mill-adenovirus tas-serotip 2.7m8 li fih il-ġene *ChrimsonR-tdTomato*	Kura tar-retinite pigmentuża
Polish	Wektor wirusowy związany z adenowirusami, serotyp 2.7m8, zawierający gen *ChrimsonR-tdTomato*	Leczenie retinopatii barwnikowej
Portuguese	Vetor viral adeno-associado de serotipo 2.7m8 contendo o gene *ChrimsonR-tdTomato*	Tratamento da retinite pigmentosa
Romanian	Vector viral adeno-asociat de serotip 2.7m8 ce conţine gena *ChrimsonR-tdTomato*	Tratamentul retinitei pigmentare

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Vírusový vektor sérotypu 2.7m8 spojený s adenovírusom obsahujúcim gén *ChrimsonR-tdTomato*	Liečba retinitis pigmentosa
Slovenian	Adenovirusom sorodni virusni vektor serotipa 2.7m8 ki vsebuje gen *ChrimsonR-tdTomato*	Zdravljenje pigmentozne retinopatije
Spanish	Vector vírico adenoasociado del serotipo 2.7m8 que contiene el gen humano *ChrimsonR-tdTomato*	Tratamiento de la retinosis pigmentaria
Swedish	Adenoassocierad virusvektor av serotyp 2.7m8, innehållande *ChrimsonR-tdTomato* genen	Behandling av retinitis pigmentosa
Norwegian	Adenoassosiert virusvektor serotype 2.7m8 som inneholder *ChrimsonR-tdTomato* genet	Behandling av retinitis pigmentosa
Icelandic	Adenóveiru tengd veiruferja af sermisgerð 2.7m8 sem inniheldur *ChrimsonR-tdTomato* gen	Meðferð á retinitis pigmentosa