



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

6 January 2017
EMA/758036/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant self-complementary adeno-associated viral vector serotype 9 containing the human *CLN3* gene for the treatment of neuronal ceroid lipofuscinosis

On 12 December 2016, orphan designation (EU/3/16/1806) was granted by the European Commission to Ser-mes Planificación SL, Spain, for recombinant self-complementary adeno-associated viral vector serotype 9 containing the human *CLN3* gene (also known as ABO-201) for the treatment of neuronal ceroid lipofuscinosis.

What is neuronal ceroid lipofuscinosis?

Neuronal ceroid lipofuscinosis is a group of inherited diseases where substances known as lipofuscins build up in the brain and other parts of the body, such as the eye. Symptoms of the disease include delayed speech, inability to coordinate muscle movements, fits, loss of vision, mental deterioration and dementia.

Neuronal ceroid lipofuscinosis is a debilitating and life-threatening condition that leads to death in early adulthood.

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

At the time of designation, neuronal ceroid lipofuscinosis affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 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 orphan designation there were no satisfactory treatments authorised in the EU for neuronal ceroid lipofuscinosis. The disease was managed by treating its symptoms.

*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).



How is this medicine expected to work?

The medicine consists of a virus that contains a copy of the fully working *CLN3* gene. This gene is defective in some patients with neuronal ceroid lipofuscinosis and this is the reason for the build-up of lipofuscins. When injected into patients, the virus is expected to carry the working *CLN3* gene into the patient's cells including brain cells. This is expected to help reduce the build-up of lipofuscins in the brain and prevent further brain damage.

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 the medicine in patients with neuronal ceroid lipofuscinosis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for neuronal ceroid lipofuscinosis 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 4 November 2016 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:

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant self-complementary adeno-associated viral vector serotype 9 containing the human <i>CLN3</i> gene	Treatment of neuronal ceroid lipofuscinosis
Bulgarian	Рекомбинантен самокомплементарен адено-асоцииран вирусен вектор серотип 9, съдържащ човешки ген <i>CLN3</i>	Лечение на липофусциноза на невроните
Croatian	Rekombinantni samodopunjavajući adeno-vezani virusni vektor serotipa 9 koji kodira ljudski <i>CLN3</i> gen	Liječenje neuronske ceroidne lipofuscinoze
Czech	Rekombinantí samokomplementární adeno-asociovaný virální vektor sérotypu 9 nesoucí lidský gen <i>CLN3</i>	Léčba neuronálního ceroidu lipofuscinózy
Danish	Rekombinant selvkomplementær adenoassocieret viral vektorserotype 9, der indeholder det humane <i>CLN3</i> gen	Behandling af neuronal ceroid lipofuscinosis
Dutch	Recombinant zelf-complementair adenogeassocierde virale vector serotype 9 welke het humane <i>CLN3</i> transgeen bevat	Behandeling van neuronale ceroidlipofuscinose
Estonian	Inimese <i>CLN3</i> geeni sisaldav rekombinantne isetäiendav adenoassotsieerunud viirusvektori 9. serotüüp	Neuronaalse tseroidse lipofustsinoosi ravi
Finnish	Rekombinantti, itseään kohti takaisin taittuva adenoassosioitu virusvektori, serotyyppiä 9, joka sisältää ihmisen <i>CLN3</i> -geenin	Neuronaalisen seroidilipofuskinoosin hoito
French	Sérotipe 9 du vecteur viral adéno-associé recombinant simple-brin complémentaire contenant le gène humain <i>CLN3</i>	Traitement des céroïdes-lipofuscinoses neuronales
German	Rekombinanter selbstkomplentärer adeno-assoziiierter viraler Vektor Serotyp 9, der das menschliche <i>CLN3</i> -Gen enthält	Behandlung von neuronaler Ceroid-Lipofuszinose
Greek	Ανασυνδυασμένος αυτοσυμπληρωματικός αδενο-σχετιζόμενος ιικός φορέας οροτύπου 9 που περιέχει το ανθρώπινο γονίδιο <i>CLN3</i>	Θεραπεία της νευρωνικής κηροειδούς λιποφουσκίνωσης
Hungarian	Humán <i>CLN3</i> gént tartalmazó 9-es szerotípusú rekombináns önkiegészítő adenoasszociált vírusvektor	Neuronalis ceroid lipofuscinosis kezelése
Italian	Vettore virale adeno-associato autocomplementare ricombinante del sierotipo 9 contenente il gene umano <i>CLN3</i>	Trattamento della ceroidolipofuscinosi neuronale giovanile
Latvian	Rekombinants paškomplementārs adeno-asociētā vīrusa vektora 9. serotips, kas satur cilvēka <i>CLN3</i> gēnu	Neironu ceroidās lipofuscinozes ārstēšana

¹ At the time of designation

Language	Active ingredient	Indication
Lithuanian	Rekombinantinis, pats sau komplimentarus, su adeno virusu susieto 9 serotipo virusinis vektorius, turintis žmogaus <i>CLN3</i> geną	Neuronų ceroidinės lipofuscinozės gydymas
Maltese	Vettur virali rikombinanti awtokomplimentari assoċjat ma' adeno ta' serotip 9 li jikkodifika l-gene uman <i>CLN3</i>	Kura ta' lipofuscinoži ċerojde Newronali
Polish	Rekombinowany samo-komplementarny wektor adenowirusa serotypu 9 zawierający ludzki gen <i>CLN3</i>	Leczenie ceroidlipofuscynozy neuronalnej
Portuguese	Vetor viral adeno-associado de sorotipo 9 auto-complementar recombinante contendo o gene humano <i>CLN3</i>	Tratamento da Lipofuscinose Ceroide neuronal
Romanian	Vector viral adeno-asociat recombinant autocomplementar de serotip 9 ce conține gena umană <i>CLN3</i>	Tratamentul lipofuscinozei ceroide neuronale
Slovak	Rekombinantný samo-komplementárny adeno-asociovaný vírusový vektor sérotypu 9 obsahujúci ľudský gén <i>CLN3</i>	Liečba neurónovej ceroidnej lipofuscinózy
Slovenian	Rekombinantni samo-dopolnilni adeno-asocirani virusni vektor serotipa 9, ki kodira človeški <i>CLN3</i> gen	Zdravljenje nevronske ceroidne lipofuscinoze
Spanish	Vector viral adenoasociado autocomplementario recombinante del serotipo 9 que contiene el gene humano <i>CLN3</i>	Tratamiento de ceroidlipofuscinosis neuronal
Swedish	Rekombinant, självkomplementär adenoassocierad virusvektor av serotyp 9 innehållandeden humana <i>CLN3</i> -genen	För behandling av neuronal ceroidlipofuscinosis
Norwegian	Rekombinant selvkomplementær adenoassosiert virusvektor serotype 9 som inneholder det humane <i>CLN3</i> -genet	Behandling av nevronal ceroid lipofuscinose
Icelandic	Raðbrigða sjálffyllandi eitlatengd veirufurja af sermisgerð 9 sem kóðar fyrir manna <i>CLN3</i> geni	Meðferð á tauga ceróið lípófuscinósis