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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase for the treatment of GM1 gangliosidosis

On 20 March 2017, orphan designation (EU/3/17/1851) was granted by the European Commission to Lysogene, France, for adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (also known as LYS-GM101) for the treatment of GM1 gangliosidosis.

What is GM1 gangliosidosis?

GM1 gangliosidosis is an inherited disorder that causes progressive damage to the nerve cells in the brain and spinal cord.

Patients with this condition lack an enzyme called beta-galactosidase which normally breaks down several molecules, including a substance called GM1 ganglioside. Without this enzyme, GM1 ganglioside builds up in the body, particularly in the brain and spinal cord, causing progressive nerve damage. Signs and symptoms include seizures (fits), learning disabilities, muscle weakness, skeletal abnormalities and problems walking and, as the disease progresses, enlargement of the heart, liver and spleen.

GM1 gangliosidosis is a debilitating and life-threatening disease. The most severe form of the disease starts in early infancy and can lead to death in a few years.

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

At the time of designation, GM1 gangliosidosis 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 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 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU to treat GM1 gangliosidosis. Treatment of patients was mainly supportive and included surgery and medicines to manage seizures, heart problems and infections.

How is this medicine expected to work?

The medicine is made of a virus that contains the gene for beta-galactosidase, the enzyme that is missing in patients with GM1 gangliosidosis. After the medicine is injected into the patient, the virus is expected to carry the gene into the cells, enabling them to produce the missing enzyme, thus restoring the body's ability to break down GM1 ganglioside.

The type of 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 GM1 gangliosidosis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for GM1 gangliosidosis. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 February 2017 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 rh.10 expressing beta-galactosidase	Treatment of GM1 gangliosidosis
Bulgarian	Адено-асоцииран вирусен вектор, серотип rh.10, експресиращ бета-галактозидаза	Лечение на GM1 ганглиозидоза
Croatian	Adeno-vezani virusni vector serotpa rh.10 koji izražava beta galaktozidazu	Liječenje GM1 gangliozidoze
Czech	Adeno-asociovaný virový vektro serotype rh 10 exprimující beta –galaktosidázu	Léčba GM1 gangliosidozy
Danish	Adenoassocieret virus serotype rh.10 vektor, som koder for beta-galactosidase	Behandling af GM1 gangliosidosis
Dutch	Adeno-geassocieerde virale vector serotype rh.10 welke beta-galactosidase uitdrukt	Behandeling vanGM1 gangliosidose
Estonian	Beeta-galaktosidaasi ekspresseeriv adenoviirusega assotseerunud viirusvektori serotüüp rh.10	GM1 gangliosidoosi ravi
Finnish	Adenoassosioitu virusvektori, serotyyppi rh.10, joka ilmentää beeta-galaktosidaasia	GM1-gangliosidoosin hoito
French	Vecteur viral adéno-associé de sérotype rh.10 codant la beta-galactosidase	Traitement de la gangliosidose à GM1
German	Adeno-assoziierter viraler Vektor vom Serotyp rh.10, der Beta-Galaktosidase exprimiert	Behandlung der GM1 Gangliosidose
Greek	Αδενοσχετιζόμενος ιικός φορέας οροτύπου rh.10 που εκφράζει τη β-γαλακτοσιδάση	Θεραπεία της γαγγλιοσίδωσης GM1
Hungarian	Béta-galaktozidázt expresszáló rh.10 szerotípusú adeno-asszociált virus vektor	GM1 gangliozidózis kezelése
Italian	Vettore virale adeno-associato di sierotipo rh.10 che codifica per la beta galattosidasi	Trattamento della gangliosidosi GM1
Latvian	Adeno-asociētā vīrusa *rh.10* serotipa vektors, kas ekspresē bēta-galaktozidāzi	GM1 gangliozidozes ārstēšana
Lithuanian	Adenoasocijuoto viruso vektoriaus serotipas rh.10, ekspresuojantis β-galaktozidazę	GM1 gangliozidozės gydymas
Maltese	Beta-galactosidase li jesprimi serotip vettur virali assoċjat ma' adeno rh.10	Kura ta' ganglijosidoži GM1
Polish	Wektor wirusowy związany z adenowirusami serotypu rh. 10 eksprymujący beta-galaktozydazę	Leczenie gangliozydozy GM1
Portuguese	Vetor viral adeno-associado de serotipo expressando a beta-galactosidase	Tratamento da gangliosidose GM1
Romanian	Vector viral adeno-asociat de serotip rh.10 ce exprimă beta-galactozidaza	Tratamentul gangliozidozei GM1
Slovak	Adeno-asociovaný vírusový vektor sérotypu rh.10 exprimujúci beta-galaktozidázu	Liečba GM1 gangliozidózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Adeno-pridruženi virusni vector serotipa rh.10, ki kodira beta-galaktozidazo	Zdravljenje GM1 gangliozidoze
Spanish	Factor viral adenoasociado de serotipo 10 que expresa beta-galactosidasa	Tratamiento de Gangliosidosis GM1
Swedish	Adenoassocierad virusvektor serotype rh.10 som uttrycker betagalaktosidas	Behandling av GM1-gangliosidos
Norwegian	Adenoassosiert virusvektor serotype rh.10 som uttrykker beta-galaktosidase	Behandling av GM1 gangliosidose
Icelandic	Adenótengd veiruferja af sermisgerð rh.10 sem tjáir beta galaktósídasa	Meðferð á GM1 ganglíósídósis