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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA for the treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

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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 16 December 2014, orphan designation (EU/3/14/1389) was granted by the European Commission to Lysogene, France, for adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA for the treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome).

What is mucopolysaccharidosis type IIIA?

Mucopolysaccharidosis type IIIA (also known as Sanfilippo A syndrome) is an inherited disease that is caused by the lack of an enzyme called N-sulfoglucosamine sulfohydrolase (SGSH). This enzyme is needed to break down a substance in the body called heparan sulphate. Because patients with mucopolysaccharidosis type IIIA cannot break this substance down, it gradually builds up in cells in the body, particularly in the brain, and damages them. This causes a wide range of symptoms, including behavioural problems, learning disabilities, difficulty moving and sleep disturbances. The disease is usually diagnosed in children between two and six years of age.

Mucopolysaccharidosis type IIIA is a seriously debilitating and life-threatening disease because it leads to poor development of language skills and movement, hyperactivity and slow development. The disease usually leads to death during adolescence.

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

At the time of designation, mucopolysaccharidosis type IIIA affected less than 0.1 in rh.10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 people*, and is below the ceiling for orphan designation, which is 5 people in rh.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 mucopolysaccharidosis type IIIA. Bone marrow transplantation had been used to try to slow down the progression of the disease.

How is this medicine expected to work?

This medicine is made up of a virus that contains the gene for the enzyme N-sulfoglucosamine sulfohydrolase, which is missing in patients with mucopolysaccharidosis type IIIA. When it is injected directly into the brain, the virus is expected to carry the gene into the brain cells. These cells will then be able to produce the missing enzyme so that it can break down the accumulated heparan sulphate and help to relieve the symptoms of the disease.

The type of virus used in this medicine (adeno-associated virus) is modified so that it 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, clinical trials with the medicine in patients with mucopolysaccharidosis type IIIA were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for mucopolysaccharidosis type IIIA. 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 13 November 2014 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 rh.10,000 people in the EU) or insufficient returns on investment.

*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 511,rh.100,000 (Eurostat 2014).

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	Adeno-associated viral vector serotype rh.10 carrying the human N-sulfoglucosamine sulfohydrolase cDNA	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)
Bulgarian	Адено-асоцииран вирусен вектор серотип rh.10, съдържаш кДНК на човешкия ген N-сулфоглюкозамин-сулфохидролаза	Лечение на мукополизахаридоза тип IIIA (синдром на Санфилипо А)
Croatian	Adeno-povezani virusni vektor serotipa rh.10 koji nosi ljudsku cDNK za N-sulfoglukozamin sulfohidrolazu	Liječenje mukopolisaharidoze tipa IIIA (Sanfilippov A sindrom)
Czech	Sérotyp virového vektoru rh.10 spojeného s adenovirem nesoucí lidskou cDNA sulfohydrolázu N-sulfoglykosaminu	Léčba mukopolysacharidozy typu IIIA (syndrom Sanfilippo A)
Danish	Adeno-associeret viral vektor serotype rh.10 der bærer human N-sulfoglucosamin sulfohydrolase cDNA	Behandling af mucopolysaccharidose type III (Sanfilippo A syndrom)
Dutch	Adeno-geassocieerde virale vector serotype rh.10 die het menselijke N-sulfoglucosamine sulfohydrolase cDNA draagt	Behandeling van mucopolysacharidose type IIIA (Sanfilippo-A-syndroom)
Estonian	Inimese N-sulfoglükosamiini sulfohüdrolaasi cDNAd kandev adeno-assotsieeritud viirusvektori serotüüp rh.10	IIIA-tüüpi mukopolüsahharidoosi (A-tüüpi Sanfilippo sündroomi) ravi
Finnish	Adeno-assosioitunut virusvektori serotyyppi rh.10 jossa ihmisen N-sulfoglukosamiini sulfohydrolaasi cDNA:ta	Tyypin IIIA (Sanfilippo A) mukopolysakkaridoosin hoito
French	Vecteur viral adéno-associé de sérotype rh.10 portant l'ADNc de la N-sulfoglucosamine sulfohydrolase humaine	Traitement de la mucopolysaccharidose de type IIIA (maladie de Sanfilippo A)
German	Adeno-assoziierter viraler Vektor Serotyp rh.10, der humane N-Sulfoglucosamine Sulfohydrolase cDNA enthält	Behandlung der Mukopolysaccharidose Typ IIIA (Sanfilippo-Syndrom Typ A)
Greek	Αδενοσυσχετιζόμενος ιϊκός φορέας οροτύπου rh.10 φέρων το cDNA της σουλφοϋδρολάσης Ν-σουλφογλυκοζαμίνης του ανθρώπου	Θεραπεία βλεννοπολυσακχαρίδωσης, τύπου IIIA (σύνδρομο Sanfilippo A)
Hungarian	Humán N-szulfoglükozamin szulfohidroláz cDNS-t hordozó rh.10-es szerotípusú adeno-asszociált vírus vector	IIIA típusú mucopolisaccharidosis (Sanfilippo A szindróma) kezelése
Italian	Vettore virale adeno associato del sierotipo rh.10 che trasporta il cDNA della N-sulfoglucosammina sulfoidrolasi umane	Trattamento della mucopolisaccaridosi di tipo IIIA (sindrome di Sanfilippo A)
Latvian	Adenovīrusa saistītā virālā vektora rh.10. serotips, kas satur cilvēka N-sulfoglikozamīna sulfohidrolāzes kDNS	IIIA tipa mukopolisaharidozes (Sanfilipo A sindroms) ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Adeno asocijuoto viruso vektoriaus rh.10 serotipas , pernešantis žmogaus N-sulfogliukozamino sulfohidrolazės cDNR	Mukopolisacharidozės, IIIA tipo gydymas (Sanfilippo A sindromas)
Maltese	Vettur imnissel mill-adenovirus tas-serotip rh.10 li jġorr N-sulfoglucosamine sulfohydrolase cDNA uman	Kura tal-mukopolisakkaridoži tat-tip IIIA (sindrome ta' Sanfilippo tat-tip A)
Polish	Wektor adenowirusowy serotypu rh.10, zawierający cDNA ludzkiej sulfohydrolazy N-sulfoglukozaminy	Leczenie mukopolisacharydozy, typ III A (zespół Sanfilippo A)
Portuguese	Vetor viral adeno-associado de serotipo rh.10 contendo o ADNc humano da N-sulfoglucosamina sulfohidrolase	Tratamento da mucopolissacaridose, tipo IIIA (síndrome de Sanfilippo de tipo A)
Romanian	Vector viral adeno-asociat de serotip rh.10, purtător al ADNc pentru N-sulfoglucozamina sulfohidrolaza umană	Tratamentul mucopolizaharidozei de tip IIIA (sindromul Sanfilippo tip A)
Slovak	Sérotyp rh.10 vírusového vektoru spojený s adenovírusom, nesúci ľudskú cDNA pre sulfohydrolázu N-sulfoglykozamínu	Liečba mukopolysacharidózy typu III.A (Sanfilippov syndróm A)
Slovenian	Adenovirusom pridruženi virusni vektor serotipa rh.10, ki vsebuje cDNA za človeško N-sulfoglukozamin sulfohidrolazo	Zdravljenje mukopolisaharidoze vrste IIIA (sindroma Sanfilippo A)
Spanish	Vector vírico adenoasociado del serotipo rh.10 que contiene el ADNc de la N-sulfoglucosamina sulfohidrolasa humana	Tratamiento de la mucopolisacaridosis tipo IIIA (síndrome de Sanfilippo A)
Swedish	Adenovirus-associerat virusvektor, serotyp rh.10 som transporterar cDNA för mänskligt N-sulfoglukosamin-sulfohydrolas	Behandling av mukopolysackaridos typ IIIA (Sanfilippos syndrom typ A)
Norwegian	Adenoassosiert virusvektor serotype rh.10 som bærer humant N-sulfoglukosamin sulfohydrolase cDNA	Behandling av mukopolysakkaridose, type IIIA (Sanfilippos syndrom type A)
Icelandic	Adenó-tengd veirugenaferja sermisgerð rh.10 sem flytur manna N-súlfóglúkósamín súlfóhýdrólasa cDNA	Meðferð við slímsykrukvilla gerð IIIA (Sanfilippo A heilkenni)