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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene for the treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome)

On 10 June 2014, orphan designation (EU/03/14/1280) was granted by the European Commission to Cochamo Systems Ltd, United Kingdom, for autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene for the treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome).

What is mucopolysaccharidosis 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.

What is the estimated number of patients?

At the time of designation, mucopolysaccharidosis type IIIA affected approximately 0.032 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,000 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 511,100,000 (Eurostat 2014).

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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?

The medicine is made up immature bone marrow cells (called CD34+) taken from the patient. These cells are able to develop into different types of immune cells. To make this medicine, the cells are modified by a virus containing the gene for the SGSH enzyme, which is lacking in patients with mucopolysaccharidosis type IIIA, resulting in the gene being introduced into the cells. When these modified cells are transplanted back into the patient, they are expected to produce the missing enzyme throughout the body, so that it can break down the accumulated heparan sulphate and help to relieve the symptoms of the disease.

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 mucopolysaccharidosis type IIIA had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for mucopolysaccharidosis type IIIA 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 9 April 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 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	Autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene	Treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome)
Bulgarian	Автоложни CD34+ клетки, трансдуцирани с лентивирусен вектор, съдържащ човешкия ген SGSH	Лечение на мукополизахаридоза тип IIIA (синдром на Санфилипо А)
Croatian	Autologne CD34+ stanice transducirane lentivirusnim vektorom koji sadrži ljudski gen SGSH	Liječenje mukopolisaharidoze tipa IIIA (Sanfillipov A sindrom)
Czech	Autologní CD34+ buňky, transdukované s lentivirovým vektorem obsahujícím lidský gen SGSH	Léčba mukopolysacharidozy typu IIIA (syndrom Sanfilippo A)
Danish	Autologe CD34+-celler transduceret med en lentiviral vektor indeholdende det humane SGSH genet	Behandling af mucopolysaccharidose type IIIA (Sanfilippo A syndrom)
Dutch	Autologe CD34+-cellen getransduceerd met een lentivirale vector die het humane gen SGSH bevat	Behandeling van mucopolysacharidose type IIIA (Sanfilippo-A-syndroom)
Estonian	Autoloogsed CD34+ rakud, mida on transdutseeritud inimese SGSH geeni sisaldava lentiviraalse viirusektoriga	IIIA-tüüpi mukopolüsahharidoosi (A-tüüpi Sanfilippo sündroomi) ravi
Finnish	Autologisia CD34+-soluja, jotka on transdusoitu ihmisen SGSH-geenin sisältävällä lentivirusvektorilla	Tyypin IIIA (Sanfilippo A) mukopolysakkaridoosin hoito
French	Cellules autologues CD34+ transduites avec un vecteur lentiviral contenant le gène humain SGSH	Traitement de la mucopolysaccharidose de type IIIA (maladie de Sanfilippo A)
German	Autologe CD34+-Zellen mit einem lentiviralen Vektor transduziert, der das menschliche Gen SGSH enthält	Behandlung der Mukopolysaccharidose Typ IIIA (Sanfilippo-Syndrom Typ A)
Greek	Αυτόλογα CD34+ κύτταρα διαμολυσμένα με φορέα λεντοϊού που περιέχει το ανθρώπινο γονίδιο SGSH	Θεραπεία βλεννοπολυσακχαρίδωσης, τύπου IIIA (σύνδρομο Sanfilippo A)
Hungarian	Humán SGSH gént hordozó lentivírus vektorral transzdukált autológ CD34+ sejtek	IIIA típusú mucopolisacharidosis (Sanfilippo A szindróma) kezelése
Italian	Cellule CD34+ autologhe trasdotte con un vettore lentivirale contenente il gene umano SGSH	Trattamento della mucopolisaccaridosi di tipo IIIA (sindrome di Sanfilippo A)
Latvian	Autologas CD34+ šūnas pārveidotas ar lentavirusa vektoru, kas satur cilvēka SGSH gēnu	IIIA tipa mukopolisaharidozes (Sanfilipo A sindroms) ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Autologinės CD34+ ląstelės transdukuotos su lentiviruso vektoriumi, turinčiu žmogaus SGSH geną	Mukopolisacharidozės, IIIA tipo gydymas (Sanfilippo A sindromas)
Maltese	Ċelluli CD34+ awtologużi trasformati b'vettur lentivirali li fih il-ġene SGSH uman	Kura tal-mukopolisakkaridoża tat-tip IIIA (sindrome ta' Sanfilippo tat-tip A)
Polish	Autologiczne komórki CD34+ transdukowane wektorem lentiwirusowym zawierającym ludzki gen SGSH	Leczenie mukopolisacharydozy, typ III A (zespół Sanfilippo A)
Portuguese	Células CD34+ autólogas transduzidas com um vector lentiviral contendo o gene humano SGSH	Tratamento da mucopolissacaridose, tipo IIIA (síndrome de Sanfilippo de tipo A)
Romanian	Celule autologe CD34+ transduse cu un vector lentiviral conţinând gena umană SGSH	Tratamentul mucopolizaharidozei de tip IIIA (sindromul Sanfilippo tip A)
Slovak	Autológne CD34+ bunky, transdukované lentivírusovým vektorom obsahujúcim ľudský gén SGSH	Liečba mukopolysacharidózy typu IIIA (Sanfilippov syndróm A)
Slovenian	Avtologne Celice CD34+, transducirane z lentivirusnim vektorjem, ki vsebuje gen humanega SGSH	Zdravljenje mukopolisaharidoze vrste IIIA (sindroma Sanfilippo A)
Spanish	Células CD34+ autólogas transducidas con un vector lentiviral que contiene el gen humano SGSH	Tratamiento de la mucopolisacaridosis tipo IIIA (síndrome de Sanfilippo A)
Swedish	Autologa CD34+ celler transducerade med en lentiviral vektor innehållande den mänskliga SGSH genen	Behandling av mukopolysackaridos typ IIIA (Sanfilippos syndrom typ A)
Norwegian	Autologe CD34+-celler transdusert med en lentiviral vektor inneholdende humant SGSH gen	Behandling av mukopolysakkaridose, type IIIA (Sanfilippos syndrom type A)
Icelandic	Samgena CD34+ frumur sem eru veiruleiddar með lentiveiru vektor sem inniheldur manna SGSH genið	Meðferð við slímsykrukvilla gerð IIIA (Sanfilippo A heilkenni)