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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Chemically modified human recombinant sulfamidase for the treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

On 14 October 2016, orphan designation (EU/3/16/1747) was granted by the European Commission to Swedish Orphan Biovitrum AB (publ), Sweden, for chemically modified human recombinant sulfamidase 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 sulfamidase. 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 affected by the condition?

At the time of designation, mucopolysaccharidosis type IIIA 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 513,700,000 (Eurostat 2016).

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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. Patients received supportive treatment to temporarily relieve the symptoms of the disease, such as physiotherapy, speech therapy and behavioural therapy.

How is this medicine expected to work?

This medicine is a copy of the enzyme missing in patients with mucopolysaccharidosis type IIIA. The enzyme in this medicine has been modified in the laboratory so that it can enter brain cells more easily, and remains active for longer before being eliminated from the body. As a result, the cells will be able to break down the accumulated heparan sulphate, thereby helping 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 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 8 September 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	Chemically modified human recombinant sulfamidase	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)
Bulgarian	Химически модифицирана човешка рекомбинантна сулфамидаза	Лечение на мукополизахаридоза тип IIIA (синдром на Санфилипо А)
Croatian	Kemijski modificirana rekombinantna humana sulfamidaza	Liječenje mukopolisaharidoze tipa IIIA (Sanfilippov A sindrom)
Czech	chemicky modifikovaná lidská rekombinantní sulfamidáza	Léčba mukopolysacharidozy typu IIIA (syndrom Sanfilippo A)
Danish	Kemisk modificeret rekombinant human sulfamidase	Behandling af mucopolysaccharidose type III (Sanfilippo A syndrom)
Dutch	chemisch gemodificeerd humaan recombinant sulfamidase	Behandeling van mucopolysacharidose type IIIA (Sanfilippo-A-syndroom)
Estonian	Inimese keemiliselt muundatud rekombinantne sulfamidaas	IIIA-tüüpi mukopolüsahharidoosi (A-tüüpi Sanfilippo sündroomi) ravi
Finnish	Kemiallisesti muokattu ihmisen rekombinantti sulfamidaasi	Tyypin IIIA (Sanfilippo A) mukopolysakkaridoosin hoito
French	Recombinant de la sulfamidase humaine modifiée chimiquement	Traitement de la mucopolysaccharidose de type IIIA (maladie de Sanfilippo A)
German	Chemisch modifizierte, humane, rekombinante Sulfamidase	Behandlung der Mukopolysaccharidose Typ IIIA (Sanfilippo-Syndrom Typ A)
Greek	Χημικά τροποποιημένη ανασυνδυασμένη ανθρώπινη σουλφαμιδάση	Θεραπεία βλεννοπολυσακχαρίδωσης, τύπου IIIA (σύνδρομο Sanfilippo A)
Hungarian	Kémiailag módosított humán rekombináns szulfamidáz	IIIA típusú mucopolisaccharidosis (Sanfilippo A szindróma) kezelése
Italian	Sulfamidasi umana ricombinante chimicamente modificata	Trattamento della mucopolisaccaridosi di tipo IIIA (sindrome di Sanfilippo A)
Latvian	Ķīmiski modificēta cilvēka rekombinantā sulfamidāze	IIIA tipa mukopolisaharidozes (Sanfilipo A sindroms) ārstēšana
Lithuanian	Chemiškai modifikuota rekombinantinė žmogaus sulfamidazė	Mukopolisacharidozės, IIIA tipo gydymas (Sanfilippo A sindromas)
Maltese	Sulfamidase rikombinanti uman modifikat b'mod kimiku	Kura tal-mukopolisakkaridoži tat-tip IIIA (sindrome ta' Sanfilippo tat-tip A)
Polish	Chemicznie modyfikowana rekombinowana ludzka sulfamidaza	Leczenie mukopolisacharydozy, typ III A (zespół Sanfilippo A)
Portuguese	Sulfamidase humana recombinante quimicamente modificada	Tratamento da mucopolissacaridose, tipo IIIA (síndrome de Sanfilippo de tipo A)
Romanian	Sulfamidază umană recombinantă, modificată chimic	Tratamentul mucopolizaharidozei de tip IIIA (sindromul Sanfilippo tip A)
Slovak	Chemicky modifikovaná ľudská rekombinantná sulfamidáza	Liečba mukopolysacharidózy typu III.A (Sanfilippov syndróm A)

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	kemično modificirana rekombinantna humana sulfamidaza	Zdravljenje mukopolisaharidoze vrste IIIA (sindroma Sanfilippo A)
Spanish	Proteína sulfamidasa recombinante de origen humano químicamente modificada	Tratamiento de la mucopolisacaridosis tipo IIIA (síndrome de Sanfilippo A)
Swedish	Kemiskt modifierat humant rekombinant sulfamidase	Behandling av mukopolysackaridos typ IIIA (Sanfilippos syndrom typ A)
Norwegian	Kjemisk modifisert human rekombinant sulfamidase	Behandling av mukopolysakkaridose, type IIIA (Sanfilippos syndrom type A)
Icelandic	Efnafræðilega breyttur manna súlfamídasi , sem framleiddur er með raðbrigða erfðatækni	Meðferð við slímsykrukvilla gerð IIIA (Sanfilippo A heilkenni)