



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Public summary of opinion on orphan designation

Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside for the treatment of anti-MAG neuropathy

On 17 July 2017, orphan designation (EU/3/17/1893) was granted by the European Commission to SFL Regulatory Affairs Consulting Ltd, United Kingdom, for polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside for the treatment of anti-MAG neuropathy.

### What is anti-MAG neuropathy?

Anti-MAG neuropathy is a progressive disease of the peripheral nerves (nerves outside the brain and spinal cord). In this disease, the body's immune (defence) system produces antibodies that incorrectly target and destroy a natural substance called myelin-associated glycoprotein (MAG). MAG is an important part of the protective sheath around the nerve cells in the body. The condition typically occurs in people aged above 50 years and is more common in men than in women. Patients with the condition lose sensation first in their fingers and toes and go on to develop muscle weakness, lack of coordination, tremors and nerve pain. As the condition worsens, patients need nursing support and become wheelchair bound.

Anti-MAG neuropathy is debilitating in the long term because of increasing inability to control muscle movement and loss of sensation. The condition is not however considered life-threatening.

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

At the time of designation, anti-MAG neuropathy affected approximately 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 119,000 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).

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\*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).



## **What treatments are available?**

At the time of designation, no satisfactory methods were authorised for the treatment of anti-MAG neuropathy. Treatment included medicines to reduce the activity of the immune system, and procedures to remove antibodies from the blood.

## **How is this medicine expected to work?**

This medicine attaches to MAG antibodies and stops them attaching to MAG. By neutralising MAG antibodies in this way, the medicine is expected to prevent the immune system from attacking MAG. This is expected to stop damage to the protective sheath around nerve cells and prevent progression of anti-MAG neuropathy.

## **What is the stage of development of this medicine?**

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with anti-MAG neuropathy had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for anti-MAG neuropathy 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 15 June 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside	Treatment of anti-MAG neuropathy
Bulgarian	Полифенил(двунариев 3-О-сулфо-бета-D-глюкопирануронат)-(1→3)-бета-D-галактопиранозид	Лечение на анти-MAG невропатия
Croatian	Polifenil (disodium 3-O-sulfo-beta-D-glukopiranonat)-(1→3)-beta-D-galaktopiranozid	Liječenje anti-MAG neuropatije
Czech	Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranosid	Léčba anti-MAG polyneuropatie
Danish	Polyphenyl(dinatrium-O-sulfo-beta-D-glucopyranuronat)-(1→3)-beta-D-galactopyranosid	Behandling af anti-MAG neuropati
Dutch	Polyfenyl(dinatrium 3-O-sulfo-beta-D-glucopyranuronaat)-(1→3)-beta-D-galactopyranoside	Behandeling van anti-MAG neuropathie
Estonian	Polüfenüül(dinaatrium 3-O-sulfo-beeta-D-glükopüranuronaat)-(1→3)-beeta-D-galaktopüranosiid	Anti-MAC neuropaatia (müeliiniga seotud glükoproteiinivastaste antikehadega neuropaatia) ravi
Finnish	Polyfenyyli (disodium 3-O-sulfo-beta-D-glukopyranuronaatti)-(1→3)-beta-D-galaktopyranosidi	Anti-MAG-neuropatian hoito
French	Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside	Traitement de la neuropathie anti-MAG
German	Polyphenyl(dinatrium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranosid	Behandlung der Anti-MAG Neuropathie
Greek	Πολυφαινυλ (δινατριούχο 3-Ο-σουλφο-β-D-γλυκοπιρανουρονικό)-(1→3)-β-D-γαλακτοπιρανοσίδιο	Θεραπεία της αντι-MAG νευροπάθειας
Hungarian	Polifenil(disodium 3-O-szolfo-béta-D-glukopiranonát)-(1→3)-béta-D-galaktopiranozid	Anti-MAG neuropátia kezelése
Italian	Poifenil(disodio 3-O-sulfo-beta-D-glucopiranonato)-(1→3)-beta-D-galattopiranoside	Trattamento della neuropatia anti-MAG
Latvian	Polifenil(dinātrija 3-O-sulfo-beta-D-glikopiranuronāta)-(1→3)-beta-D-galaktopiranozīds	Anti-MAG neiropātijas ārstēšana

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Lithuanian	Polifenil (dinatrio 3-O-sulfo-beta-D-glukopiranonatas) (1→3)-beta-D-galaktopiranozidas	Anti-MAG neuropatijos gydymas
Maltese	Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside	Kura ta' newropatija anti-MAG
Polish	Polifenyl(disodo 3-O-sulfo-beta-D-glukopiranonat)-(1→3)-beta-D-galaktopiranozyd	Leczenie neuropatii z przeciwciałami anti-MAG
Portuguese	Polifenil(3-O-sulfo-beta-D-glucopiranonato dissódico)-(1→3)-beta-D-galactopiranosido	Tratamento da neuropatia anti-MAG
Romanian	Polifenil (disodium 3-O-sulfo-beta-D-glucopiranonat)-(1→3)-beta-D-galaktopiranozid	Tratamentul neuropatiei anti-MAG
Slovak	Polyfenyl(disodný 3-O-sulfo-beta-D-glukopyranuronát)-(1→3)-beta-D-galaktopyranozid	Liečba anti-MAG neuropatie
Slovenian	Polifenil(dinatrijev 3-O-sulfo-beta-D-glukopiranonat)-(1→3)-beta-D-galaktopiranozid	Zdravljenje anti-MAG nevropatije
Spanish	Polifenil(3-O-sulfo-beta-D-glucopiranonato dissódico)-(1→3)-beta-D-galactopiranosido	Tratamiento de la Anti GAM neuropatia periferica
Swedish	Polyfenyl(di-natrium 3-O-sulfo-beta-D-glucopyranuronat)-(1→3)-beta-D-galactopyranosid	Behandling av anti-MAG-neuropati
Norwegian	Polyfenyl(dinatrium 3-O-sulfo-beta-D-glukopyranuronat)-(1→3)-beta-D-galaktopyranosid	Behandling av anti-MAG nevropati
Icelandic	Pólýfenýl(dínatríum 3-D-súlfó-beta-D-glúkópýra núróat)-(1-3)-beta-D-galaktópýranós+ið	Meðferð á anti-MAG neuróþathíu