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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Sodium chlorite for the treatment of amyotrophic lateral sclerosis

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On 19 June 2013, orphan designation (EU/3/13/1139) was granted by the European Commission to Shore Limited, United Kingdom, for sodium chlorite (also known as NP001) for the treatment of amyotrophic lateral sclerosis.

### What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate. This causes loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS vary depending on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, difficulty speaking, swallowing and breathing. ALS usually starts in mid-life and men are more likely to develop the disease than women.

ALS is a long-term debilitating and life-threatening disease because of the gradual loss of function and its paralysing effect on muscles used for breathing which usually leads to death due to respiratory failure.

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

At the time of designation, ALS affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 people*, and is below the ceiling for orphan designation,

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
**Telephone** +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
**E-mail** info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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).

## What treatments are available?

At the time of designation, medicines authorised in the EU to treat ALS included riluzole. Patients also received supportive treatment to temporarily relieve the symptoms of the disease, such as physiotherapy and speech therapy.

The sponsor has provided sufficient information to show that sodium chlorite might be of significant benefit for patients with ALS because early studies in preclinical models show that it might delay the progression of the disease. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

## How is this medicine expected to work?

Sodium chlorite interferes with the functions of macrophages, a type of white blood cell that forms part of the immune system. Macrophages are involved in the inflammation process. In ALS, they are believed to be over-activated, producing high levels of substances called cytokines which attack and damage the nerve cells in the brain and spinal cord. By blocking the activity of the macrophages, sodium chlorite is expected to reduce their activity, thereby preventing the progression of the disease. The medicine is expected to be given by injection into a vein.

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

The effects of sodium chlorite have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sodium chlorite in patients with ALS were ongoing.

At the time of submission, sodium chlorite was not authorised anywhere in the EU for ALS. Orphan designation of sodium chlorite had been granted in the United States of America for slowing progression of ALS.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 May 2013 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:

Shore Limited
41 Tolmers Road
Cuffley
Herts EN6 4JG
United Kingdom
Telephone: +44 170 7879 828
Telefax: +44 870 7065 335
E-mail: sue@shore.ltd.uk

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 | Sodium chlorite | Treatment of amyotrophic lateral sclerosis |
| Bulgarian | Натриев хлорит | Лечение на амиотрофична латерална склероза |
| Czech | Chloritan sodný | Léčba amyotrofické laterální sklerózy |
| Danish | Natriumchlorit | Behandling af amyotrofisk lateralsklerose |
| Dutch | Natriumchloriet | Behandeling van amyotrofe lateraalsclerose |
| Estonian | Naatriumkloriit | Amüotroofilise lateraalskleroosi ravi |
| Finnish | Natriumkloriitti | Amyotrofisen lateraaliskleroosin hoito |
| French | Chlorite de sodium | Traitement de la sclérose latérale amyotrophique |
| German | Natriumchlorit | Behandlung der amyotrophen Lateralsklerose |
| Greek | Χλωρικό νάτριο | Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης |
| Hungarian | Nátrium-klorit | Amyotrophiás lateral sclerosis kezelése |
| Italian | Clorito di sodio | Trattamento della sclerosi laterale amiotrofica |
| Latvian | Nātrija hlorīds | Amiotrofiskās laterālās sklerozes ārstēšana |
| Lithuanian | Natrio chloritas | Šoninės amiotrofinės sklerozės gydymas |
| Maltese | Sodium chlorite | Kura tas-sklerosi laterali amjotrofika |
| Polish | Sodu chloryn | Leczenie stwardnienia bocznego zanikowego |
| Portuguese | Clorito de sódio | Tratamento da esclerose lateral amiotrófica |
| Romanian | Clorit de sodiu | Tratamentul sclerozei laterale amiotrofice |
| Slovak | Chloritan sodný | Liečba amyotrofickej laterálnej sklerózy |
| Slovenian | Natrijev klorit | Zdravljenje amiotrofične lateralne skleroze |
| Spanish | Clorito de sodio | Tratamiento de la esclerosis lateral amiotrófica |
| Swedish | Natriumklorit | Behandling av amyotrofisk lateralskleros |
| Norwegian | Natriumkloritt | Behandling av amyotrofisk lateralsklerose |
| Icelandic | Natríumklórít | Meðferð við blandaðri hreyfitaugahrörnun |

[1] At the time of designation