



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

3-pentylbenzeneacetic acid sodium salt for the treatment of Alström syndrome

On 12 January 2017, orphan designation (EU/3/16/1810) was granted by the European Commission to ProMetic Pharma SMT Limited, United Kingdom, for 3-pentylbenzeneacetic acid sodium salt for the treatment of Alström syndrome.

What is Alström syndrome?

Alström syndrome is a genetic disease that causes a variety of problems in several organs across the body. Signs and symptoms first occur in infancy and include vision impairment, hearing loss, obesity, diabetes, and problems with the heart, liver, kidneys and lungs.

The condition is caused by a defect in a gene (ALMS1), which produces a protein thought to play a role in the function and growth of many types of cells. Alström syndrome is inherited recessively (meaning that both parents must have the defective gene for a child to have the condition).

The condition is debilitating due to its wide-ranging effects and life threatening because it can lead to multiple organ failure. Life expectancy is lower than normal, with patients rarely living beyond 50 years.

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

At the time of designation, Alström syndrome affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 500 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).

What treatments are available?

At the time of orphan designation, there were no satisfactory treatments for Alström syndrome authorised in the EU. There were clinical guidelines for managing all the complications of this disease.

*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).



How is this medicine expected to work?

This medicine works via two receptors that are involved in several body processes and are found in organs such as the pancreas, brain, heart, kidneys, liver and lungs. Early studies indicate that the medicine may have an effect in reducing some signs and symptoms of the disease including those linked to diabetes and scarring of the liver.

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 Alström syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for Alström syndrome 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 December 2016 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	3-pentylbenzeneacetic acid sodium salt	Treatment of Alström syndrome
Bulgarian	Натриева сол на 3-пентилбензеноцетна киселина	Лечение на синдрома на Алстръм
Croatian	Natrijev 3-pentilfenilacetat	Liječenje Alströmovog sindroma
Czech	sodná sůl kyseliny 3-pentylbenzenoctové	Léčba Alströмова syndromu
Danish	3-pentylbenzenacetylsyre natriumsalt	Behandling af Alström syndrom
Dutch	3-pentylbenzeenazijnzuur natriumzout	Behandeling van het syndroom van Alström
Estonian	3-pentüülbenseenäädikhappe naatriumisool	Alströmi sündroomi ravi
Finnish	3-pentyyli-fenyylietikkahapon natriumsuola	Alströmin oireyhtymän hoito
French	Sel sodique d'acide 3-pentylbenzèneacétique	Traitement du syndrome d'Alström
German	3-Pentylbenzoesäure-Natriumsalz	Behandlung des Alström-Syndroms
Greek	Άλας του 3-πεντυλβενζολοξικού οξέος με νάτριο	Θεραπεία του συνδρόμου Alström
Hungarian	3-pentyl-benzene ecetsav nátriumsó	Alström szindróma kezelésére
Italian	Acido 3-pentilbenzenacetico, sale sodico	Trattamento della sindrome di Alström
Latvian	3-pentilfeniletiķskābes nātrija sāls	Alstrēma (<i>Alström</i>) sindroma ārstēšana
Lithuanian	3-pentilbenzeno acto rūgšties natrio druska	<i>Alström</i> sindromo gydymas
Maltese	3-pentylbenzeneacetic acid sodium salt	Kura tas-sindromu ta' Alström
Polish	Sól sodowa kwasu 3-pentylbenzenoocetowego	Leczenie zespołu Alströma
Portuguese	Sal sódico de ácido 3-pentilbenzenoacético	Tratamento da síndrome de Alström
Romanian	Sare de sodiu a acidului 3-pentilbenzenacetice	Tratamentul sindromului Alström
Slovak	Sodná soľ kyseliny 3-pentylbenzénoctovej	Liečbu Alströmovho syndrómu
Slovenian	3-pentil-benzenocetna kislina, natrijeva sol	Zdravljenje Alströmovega sindroma
Spanish	Ácido 3-pentilfenilacético, sal sódica	Tratamiento del síndrome de Alström
Swedish	3-pentylbensenättiksyra, natriumsalt	Behandling av Alströms syndrom
Norwegian	3-pentylbenzen eddiksyre natriumsalt	Behandling av Alstrøm syndrom
Icelandic	3-pentýlbensenediksyra, natríumsalt	Til meðferðar á Alström-heilkenni

¹ At the time of designation