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EMA/5008/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

[5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphine]manganese(III) chloride for the treatment of Cockayne syndrome

On 12 January 2017, orphan designation (EU/3/16/1809) was granted by the European Commission to Institut Pasteur, France, for [5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphine]manganese(III) chloride (also known as MnTBAP) for the treatment of Cockayne syndrome.

What is Cockayne syndrome?

Cockayne syndrome is an inherited disease that leads to failure to gain weight and grow, short stature, small head size (microcephaly) and delayed learning. The signs and symptoms of this condition are usually apparent at a very young age and worsen over time. Other features include severe sunburn-like reactions following exposure to light (photosensitivity), hearing loss, loss of vision, tooth decay, bone abnormalities and changes in the brain that can be seen on brain scans.

Cockayne syndrome is a seriously debilitating disease due to developmental delay and severe disability it causes. It is also life threatening with people affected living not longer than 12 years.

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

At the time of designation, Cockayne syndrome affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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 designation, there were no treatments for Cockayne syndrome. Patients mainly received supportive treatment including physical therapies such as physiotherapy, occupational and speech therapy, and hearing aids, cataract surgery and protection against UV radiation.

^{*}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 is expected to neutralise harmful substances known as toxic oxygen free radicals (ROS) and reactive nitrogen species (RNS) which cause cell damage in patients with Cockayne syndrome. By neutralising these substances, the medicine is expected to help reduce cell damage and improve the patient's condition.

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 Cockayne syndrome had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for Cockayne 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	[5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphine]manganese(III) chloride	Treatment of Cockayne syndrome
Bulgarian	[5,10,15,20-тетраakis(4-карбокисфенил)-21H,23H-порфин]манганов(III) хлорид	Лечение на синдрома на Кокейн
Croatian	[5,10,15,20-tetrakis(4-karboksifenil)-21H,23H-porfin]mangan(III) klorid	Liječenje sindroma Cockayne
Czech	[5,10,15,20-tetrakis(4-karboxyfenyl)-21H,23H-porfin]mangan(III) chlorid	Léčba Cockaynova syndromu
Danish	[5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphin]mangan(III) chlorid	Behandling af Cockayne-syndrom
Dutch	[5,10,15,20-tetrakis(4-carboxyfenyl)-21H,23H-porfine]mangaan(III)chloride	Behandeling van het syndroom van Cockayne
Estonian	[5,10,15,20-tetrakis (4-karboksüfenüül) -21H, 23H-porfüriin]mangaan (III) kloriid	Cockayne'i sündroomi ravi
Finnish	[5,10,15,20-tetrakis(4-karboksyylifenyyli)-21H,23H-porfiini]mangaani(III)kloridi	Cockaynen oireyhtymän hoito
French	Chlorure de manganèse (III)-[5,10,15,20-tétrakis(4-carboxyphényl)-21H,23H-porphine]	Traitement du syndrome de Cockayne
German	[5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphin]-mangan(III)-chlorid	Behandlung des Cockayne-Syndroms
Greek	[5,10,15,20-τετράakis(4-καρβοξυφαινυλο)-21H,23H-πορφίνη] χλωριούχο μαγγάνιο(III)	Θεραπεία του Συνδρόμου Cockayne
Hungarian	[5,10,15,20-tetrakis(4-karboxifenil)-21H,23H-porfin]mangán(III)-klorid	Cockayne-szindróma kezelésére
Italian	Cloruro di [5,10,15,20-tetrakis(4-carbossifenil)-21H,23H-porfina]manganese(III)	Trattamento della sindrome di Cockayne
Latvian	[5,10,15,20-tetrakis(4-karboksifenil)-21H,23H-porfina]mangāna(III) hlors	Kokeina (<i>Cockayne</i>) sindroma ārstēšana
Lithuanian	[5,10,15,20-tetrakis(4-karboksifenil)-21H,23H-porfin]mangano (III) chloridas	<i>Cockayne</i> sindromo gydymas
Maltese	[5,10,15,20-tetrakis(4-carboxyphenyl)-21H,23H-porphine]manganese(III) klorur	Kura tas-sindromu ta' Cockayne
Polish	chlerek manganu(III)[5,10,15,20-tetrakis(4-karboksyfenylo)-21H,23H-porfiny]	Leczenie zespołu Cockayne'a
Portuguese	Cloreto de [5,10,15,20-tetrakis(4-carboxifenil)-21H,23H-porfina]mangânês(III)	Tratamento da síndrome de Cockayne
Romanian	Clorură de [5,10,15,20-tetrakis(4-carboxifenil)-21H,23H-porfină]mangan(III)	Tratamentul sindromului Cockayne
Slovak	[5,10,15,20-tetrakis(4-karboxyfenyl)-21H,23H-porfin]mangán(III) chlorid	Liečba Cockaynovho syndrómu

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	[5,10,15,20-tetrakis(4-karboksifenil)-21H,23H-porfin]mangan(III) klorid	Zdravljenje sindroma Cockayne
Spanish	[5,10,15,20-tetrakis(4-carboxifenil)-21H,23H-porfina] cloruro de manganeso (III)	Tratamiento del síndrome de Cockayne
Swedish	[5,10,15,20-tetrakis(4-karboxyfenyl)-21H,23H-porfin]mangan(III) klorid	Behandling av Cockaynes syndrom
Norwegian	[5,10,15,20-tetrakis(4-karboksyfenyl)-21H,23H-porfin]mangan(III)-klorid	Behandling av Cockaynes syndrom
Icelandic	[5,10,15,20-tetrakis(4-karboxýfenýl)-21H,23H-porfín]mangan(III) klóríð	Til meðferðar við Cockayne-heilkenni