

10 November 2014
EMA/COMP/552795/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cysteamine hydrochloride for the treatment of cystinosis

On 15 October 2014, orphan designation (EU/3/14/1341) was granted by the European Commission to Lucane Pharma SA, France, for cysteamine hydrochloride for the treatment of cystinosis.

What is cystinosis?

Cystinosis is an inherited disease in which the amino acid cystine builds up within cells. The cystine forms crystals that can damage the organs, especially the kidneys and the eyes. Cystinosis is caused by abnormalities in a protein called cystinosin, which normally helps to remove excess cystine from cells.

Cystinosis is a long-term debilitating condition that can lead to sight loss and intolerance to light and which may be life threatening because it can lead to kidney failure if left untreated.

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

At the time of designation, cystinosis affected approximately 0.15 in 10,000 people in the European Union (EU). This was equivalent to a total of around 7,600 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 application for orphan designation, two medicines (Cystagon and Procysbi) containing cysteamine were authorised in the EU for use by mouth in the treatment of cystinosis. Ophthalmic (eye) formulations of cysteamine were not authorised in the EU, but were made up locally and used for the management of eye symptoms.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with cystinosis because the existing authorised medicines containing cysteamine

*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 511,100,000 (Eurostat 2014).

given by mouth cannot adequately treat deposits of cystine in the eyes, whereas results from published studies suggest that this medicine, which is formulated as eye drops, can reduce such deposits. 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?

Cysteamine hydrochloride works by reacting with cystine to form other substances that are able to be removed from the cells. As a result, the amount of cystine in the cells is reduced, limiting the amount of organ damage.

What is the stage of development of this medicine?

The sponsor has provided data in experimental models from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, no clinical trials with this medicine in patients with cystinosis had been started by the sponsor.

At the time of submission, the medicine was not authorised anywhere in the EU for cystinosis 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 4 September 2014 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:

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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	Cysteamine hydrochloride	Treatment of cystinosis
Bulgarian	Цистеамин хидрохлорид	Лечение на цистинозата
Croatian	Cisteaminoklorid	Liječenje cistinoze
Czech	Mercaptaminum hydrochloridum	Léčba cystinózy
Danish	Cysteamin hydroklorid	Behandling af cystinose
Dutch	Cysteaminehydrochloride	Behandeling van cystinose
Estonian	Tsüsteamiin-vesinikkloriid	Tsüstinoosi ravi
Finnish	Kysteamiinihydrokloridi	Kystinoosin hoito
French	Chlorhydrate de cystéamine	Traitement de la cystinose
German	Cysteaminhydrochlorid	Behandlung der Zystinose
Greek	Υδροχλωρική κυστεαμίνη	Θεραπεία της κυστίνωσης
Hungarian	Ciszteamin hidroklorid	Cystinosis kezelése
Italian	Cisteamina cloridrato	Trattamento della cistinosi
Latvian	Cisteamīna hidrohlorīds	Cistinozes ārstēšana
Lithuanian	Cisteamino hidrochloridas	Cistinozės gydymas
Maltese	Cysteamine hydrochloride	Kura taċ-ċistinożi
Polish	Chlorowodorek cysteaminy	Leczenie cystynozy
Portuguese	Cloridrato de cisteamina	Tratamento da cistinose
Romanian	Clorhidrat de cisteamină	Tratamentul cistinozei
Slovak	Cysteamíniumchlorid	Liečba cystinózy
Slovenian	Cisteaminijev klorid	Zdravljenje cistinoze
Spanish	Cisteamina clorhidrato	Tratamiento de la cistinosis
Swedish	Cysteaminhydroklorid	Behandling av cystinos
Norwegian	Cysteaminhydroklorid	Behandling av cystinose
Icelandic	Cysteamín hýdróklóríð	Meðferð cystíngeymdarkvilla

¹ At the time of designation