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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cysteamine bitartrate (gastroresistant) for the treatment of cystinosis

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 20 September 2010, orphan designation (EU/3/10/778) was granted by the European Commission to Raptor Pharmaceuticals Europe BV, the Netherlands, for cysteamine bitartrate (gastroresistant) 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 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 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 people*, and is below the threshold 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).

*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.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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What treatments are available?

At the time of designation, cysteamine bitartrate was authorised in the EU for the treatment of nephropathic cystinosis. This medicine needs to be taken every six hours.

The sponsor has provided sufficient information to show that cysteamine bitartrate (gastroresistant) might be of significant benefit for patients with cystinosis because the medicine is a new formulation of cysteamine bitartrate that is expected to be given less often than the existing medicine. In addition, the new formulation may have a more pleasant odour and taste, making it easier for patients to take the medicine. These assumptions 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 bitartrate works by reacting with cystine to form other substances that can then be removed from the cells. As a result, the amount of cystine in the cells is reduced, limiting the amount of organ damage.

The gastroresistant formulation of cysteamine bitartrate allows cysteamine bitartrate to reach the intestine without being broken down in the stomach. Because cysteamine is absorbed better in the small intestine than in the stomach, this enables more cysteamine to be absorbed with the gastroresistant form than with the existing medicine. This is expected to allow patients to take it less often than the existing medicine (every 12 hours rather than every six hours).

What is the stage of development of this medicine?

The effects of cysteamine bitartrate (gastroresistant) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with cysteamine bitartrate (gastroresistant) in patients with cystinosis were ongoing.

At the time of submission, cysteamine bitartrate (gastroresistant) 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 2 June 2010 recommending the granting of this designation.

Update: Cysteamine bitartrate (gastroresistant) (Procysbi) has been authorised in the EU since 06 September 2013 for the treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure.

More information on Procysbi can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cysteamine bitartrate (gastroresistant)	Treatment of cystinosis
Bulgarian	Цистеамин битартрат (гастрорезистентен)	Лечение на цистинозата
Czech	Hydrogentartarát cysteaminu (gastrorezistentní)	Léčba cystinózy
Danish	Cysteaminbitartrat (gastroresistent)	Behandling af cystinose
Dutch	Cysteamine-bitartraat (gastro-resistent)	Behandeling van cystinose
Estonian	Tsüsteamiinbitartraat (gastroresistentne)	Tsüstinoosi ravi
Finnish	Kysteamiinibitartraatti (gastroresistantti)	Kystinoosin hoito
French	Bitartrate de cystéamine (gastro-résistant)	Traitement de la cystinose
German	Cysteamin-Bitartrat (magensaftresistent)	Behandlung der Zystinose
Greek	Διτρυγική κυστεαμίνη (γαστροανθεκτική)	Θεραπεία της κυστίνωσης
Hungarian	Ciszteamin-bitartarát (gyomornedv-ellenálló)	Cystinosis kezelése
Italian	Cisteamina bitartrato (gastroresistente)	Trattamento della cistinosi
Latvian	Cisteamīna bitartrāts (kuņģi aizsargājošs)	Cistinozes ārstēšana
Lithuanian	Cisteamino bitartratas (skrandyje neirus)	Cistinozės gydymas
Maltese	Cysteamine bitartrate (gastro-reżistenti)	Kura taċ-ċistinożi
Polish	Dwuwinian cysteaminy (dojelitowy)	Leczenie cystynozy
Portuguese	Bitartarato de cisteamina (gastroresistente)	Tratamento da cistinose
Romanian	Cisteamină bitartrat (gastrorezistentă)	Tratamentul cistinozei
Slovak	Cysteamínbitartarát (gastrorezistentný)	Liečba cystinózy
Slovenian	Cisteaminijev bitartrat (gastrorezistenten)	Zdravljenje cistinoze
Spanish	Bitartrato de cisteamina (gastroresistente)	Tratamiento de la cistinosis
Swedish	Cysteaminbitartrat (gastroresistant)	Behandling av cystinos
Norwegian	Cysteaminbitartrat (gastroresistent)	Behandling av cystinose
Icelandic	Systeamín bítartrat (magasýruþolin)	Meðferð cystíngeymdarkvilla

[1] At the time of designation