27 June 2013
EMA/CHMP/355942/2013
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Procysbi

mercaptamine

On 27 June 2013, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Procysbi, 25 mg, 75 mg, gastro-resistant capsules, hard intended for the treatment of nephropathic cystinosis. Procysbi was designated as an orphan medicinal product on 20 September 2010. The applicant for this medicinal product is Raptor Pharmaceuticals Europe B.V. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Procysbi is mercaptamine, an "other alimentary tract and metabolism" product (ATC Code: A16AA04). Mercaptamine participates within lysosomes in a thiol disulfide interchange reaction converting cystine into cysteine and cysteine-cysteamine mixed disulfide, both of which can exit the lysosome in patients with cystinosis.

The benefits with Procysbi are its ability to reduce 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. The most common side effects are gastrointestinal disorders such as vomiting, nausea, diarrhoea. Other very common side effects are: anorexia, lethargy, pyrexia.

A pharmacovigilance plan for Procysbi will be implemented as part of the marketing authorisation.

The approved indication is: "Procysbi is indicated 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". It is proposed that Procysbi be prescribed by physicians experienced in the treatment of cystinosis.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.

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made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit-to-risk balance for Procysbi and therefore recommends the granting of the marketing authorisation.