1 April 2016
EMA/227275/2016
EMEA/H/C/004038

Questions and answers

Refusal of the marketing authorisation for Dropcys (mercaptamine hydrochloride)

Outcome of re-examination

On 17 December 2015, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of the marketing authorisation for the medicinal product Dropcys, intended to prevent and treat the build-up of the amino acid cystine in the cornea (the transparent layer in front of the eye).The company that applied for authorisation in the EU is Lucane Pharma.

The applicant requested a re-examination of the opinion. After considering the grounds for this request, the CHMP re-examined the initial opinion and confirmed the refusal of the marketing authorisation on 1 April 2016.

What is Dropcys?

Dropcys is a medicine that contains the active substance mercaptamine hydrochloride (also known as cysteamine hydrochloride). It was to be available as a powder and a solvent, to make a solution for eye drops.

What was Dropcys expected to be used for?

Dropcys was expected to be used to prevent and treat cystinosis affecting the cornea. Cystinosis is a rare inherited disease caused by the accumulation of a substance called cystine, which forms crystals inside cells, particularly cells in the cornea and kidneys.

Dropcys was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 15 October 2014 for the treatment of cystinosis. More information on the orphan designation can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Medicines containing mercaptamine hydrochloride are authorised in the EU for use by mouth in the treatment of cystinosis. Ophthalmic (eye) formulations of mercaptamine are made up locally and used for the management of eye symptoms.

How was Dropcys expected to work?

The build-up of cystine crystals inside corneal cells can damage the eyes and cause serious vision problems. The active substance in Dropcys, mercaptamine hydrochloride, was expected to react with cystine to dissolve it and to form substances that could be removed from the cells. When the medicine is applied to the eye, the amount of cystine in the cells of the cornea was expected to be reduced, limiting the amount of eye damage.

What did the company present to support its application?

Because mercaptamine has been used for many years to treat cystinosis and its use is well established, the applicant presented data from the medical literature to support its application for Dropcys.

What were the CHMP's main concerns that led to the refusal?

The CHMP considered that the studies from the medical literature presented to support the application were insufficient. Although the role of mercaptamine eye drops in the treatment of corneal deposits appears sufficiently supported by the literature, current clinical recommendations and its routine use in hospital preparations, there were few data supporting the effectiveness of the concentration of mercaptamine used in Dropcys (0.1% solution). In addition, the CHMP had concerns regarding other ingredients of the medicine, their impact on long-term safety, particularly in children, and how stable and sterile the solution would be once prepared.

Therefore, the CHMP was of the opinion that the benefits of Dropcys did not outweigh its risks and recommended that it be refused marketing authorisation.

During the re-examination, the CHMP looked again at the data from the company. The Committee confirmed the opinion that the effectiveness of Dropcys in the treatment of corneal deposits had not been sufficiently demonstrated and that there were unresolved issues regarding the formulation and long-term safety and sterility of the medicine. Thus, the CHMP reaffirmed its previous recommendation that the medicine be refused marketing authorisation.

What consequences does this refusal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that there are no ongoing clinical trials or compassionate use programmes with Dropcys.