

Medicine: mercaptamine (brand name: Procysbi®) Resubmission

Chiesi Limited

The Scottish Medicines Consortium (SMC) has assessed mercaptamine for the treatment of nephropathic cystinosis. This document summarises the SMC decision and what it means for patients.

What has SMC said?

After careful consideration, SMC has not recommended mercaptamine for the treatment of nephropathic cystinosis.

SMC was unable to accept mercaptamine because the evidence provided by the company was not strong enough to satisfy the committee that it offers value for money to NHSScotland. This was despite using a more [flexible approach](#)* in the assessment, as it is for a rare condition.

What does SMC's decision mean for patients?

Mercaptamine should not normally be prescribed on the NHS in Scotland for use as described above. If a patient's healthcare professional thinks a particular patient would benefit from it, they can make a request to prescribe mercaptamine. All health boards have procedures in place to consider these requests. For further information see:

Medicines in Scotland: What's the right treatment for me?

www.healthcareimprovementscotland.org/medicinesbooklet.aspx



What is mercaptamine used for?

Mercaptamine is used to treat nephropathic cystinosis. Cystinosis is a rare genetic condition that causes the amino acid cystine, which occurs naturally in the body, to build up, causing crystals to form in organs of the body. These crystals cause damage to the organs and prevent them from working properly. Nephropathic cystinosis is the most severe form and is diagnosed in childhood. The kidneys and eyes are affected first but the crystals can go on to form in the muscles, pancreas, white blood cells and thyroid gland.

How does mercaptamine work?

Mercaptamine reacts with the excess cystine and forms a salt that the body can remove. This helps to reduce the amount of cystine crystals in the organs and the damage that they cause.

*<https://www.scottishmedicines.org.uk/how-we-decide/pace/>

How does SMC make its decision?

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland.

To do this SMC considers the following:

- Evidence from the company about how well the medicine works compared with current treatments available in Scotland, in relation to how much they will cost to buy and administer.
- Information from patient groups about the potential impact of the medicine on patients and carers.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.

When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not only those who may be treated with the medicine under consideration.

You can find more detailed information about the SMC assessment of mercaptamine by looking at the SMC Detailed Advice Document (SMC2374).

More information

The organisations below can provide more information and support for people with cystinosis and their families. SMC is not responsible for the content of any information provided by external organisations.

Cystinosis Foundation UK



<https://www.cystinosis.org.uk>



advice@cystinosis.org.uk

Metabolic Support UK



<https://www.metabolicsupportuk.org>



0845 241 2173

You can find out more about mercaptamine (Procysbi®) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



<https://www.ema.europa.eu/en>