

Consultation Report

Topic details

Title of policy or policy statement:	Trientine for Wilson disease
Programme of Care:	Women and Children
Clinical Reference Group:	Metabolic
URN:	170094P

1. Summary

This report summarises the outcome of a public consultation that was undertaken to test the policy proposal.

2. Background

Wilson disease is a rare complex disease caused by an excess of copper in the body which affects the liver, the brain and potentially most other organs in the body. Copper is present in most foods and is an essential element for humans. Excessive copper can lead to

- significant liver damage and liver failure;
- in the brain disturbances of motor function including seizures, movement disorders, psychosis and personality changes brain dysfunction as well as mental health problems which include psychosis and personality changes
- functional disturbances in the kidney
- in the cornea, brownish-yellow rings called Kayser-Fleischer rings

The first line treatment for symptomatic Wilson disease is a copper chelator, a drug that clears copper from the body. Penicillamine is licensed for this use. Up to a third of patients have an adverse reaction to penicillamine and it is not clinically appropriate to continue with this treatment. Trientine dihydrochloride, another copper chelator, is the preferred second line treatment offered to patients with symptoms of Wilson disease. Zinc salts are also an alternative option for a selected group of patients.

Worldwide, the disease affects approximately one in 30,000 individuals, giving a cohort of approximately 1854 patients in England. There is limited information in relation to the epidemiology of this disease in England. It is estimated that approximately 100 people with Wilson disease are being treated with trientine dihydrochloride.

Prior to 2015 the cost of treating patients with trientine was relatively low and was met by hospitals and primary care. The drug company increased the cost of the drug significantly in 2015/16 and in 2016/17 and this drug became excluded from the national tariff paid to hospitals. NHS England agreed to fund the cost of the drug for existing patients whilst a policy was being developed. It is thought that some patient's drug costs may still be funded through primary care.

3. Publication of consultation

The policy proposition was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 23rd August 2018 to 22nd September 2018. Consultation comments have been shared with the Policy Working Group (PWG) to enable full consideration of feedback and to support a decision on whether any changes to the policy might be recommended.

Respondents were asked the following consultation questions:

- Has all the relevant evidence been taken into account?
- Does the impact assessment fairly reflect the likely activity, budget and service impact? If not, what is inaccurate?
- Does the policy proposition accurately describe the current patient pathway that patients experience? If not, what is different?
- Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?
- Are there any changes or additions you think need to be made to this document, and why?

4. Results of consultation

There were seven responses to the consultation:

- Three patients responded
- Two clinicians
- One CCG commissioner
- A drug company who are proposing to market a product a similar product that is a new entrant to the market in England

The majority of respondents were supportive of the policy; one patient was concerned about the impact of the policy on their access to the drug from their GP and was confused about the proposed policy i.e. that it will improve access for patients to this drug. The drug company highlighted that their product was not considered as a comparator for trientine. The evidence review was completed before this drug, trientine tetrahydrochloride, was marketed in England and this drug is not yet available in England. The drug company also queried the patient numbers; however, these are based on the best information available to NHS England.

4.1 Evidence

In relation to consideration of all relevant evidence, one respondent, a drug company,

commented that the PWG should have considered the references set out below in relation to its product.

Ala A, Walker AP, Ashkan K et al. Wilson's disease. Lancet. 2007 Feb 3;369(9559):397-408.

Dzieżyc K, Karliński M, Litwin T, et al. Compliant treatment with anti-copper agents prevents clinically overt Wilson's disease in pre-symptomatic patients. Eur J Neurol. 2014 Feb;21(2):332-7.

Todd S. Refrigerated medicinal products: what pharmacists need to know. The Pharmaceutical Journal, 1 October 2008. Available at: <https://www.pharmaceutical-journal.com/learning/learning-article/refrigerated-medicinal-products-what-pharmacists-need-to-know/10036090.article?firstPass=false> Accessed 18 September, 2018

Specialist Pharmacy Service (SPS). Refrigerated storage trientine (Univar) capsules. Published 26th Jan 2016, updated 2nd Feb 2018. Available at: <https://www.sps.nhs.uk/medicines/trientine/> Accessed 18 September, 2018.”

The PWG confirmed that it has fully assessed the evidence in line with the NHS England prescribed approach and that the references outlined above do not impact on the policy proposition.

4.2 Impact assessment

Two respondents commented on the impact assessment; one respondent considered it difficult to understand. The drug company raised a number of queries regarding the assumptions in relation to patient numbers, the application of value added tax (VAT), the financial basis of the impact assessment and whether their drug would be included in the policy and at what level of recompense would be considered. As a consequence the VAT calculation was amended. The PWG confirmed that the aforementioned drug would not be considered as part of this policy proposition of the Integrated Impact Assessment.

4.3 Patient pathway

All but one respondent said that the policy accurately reflected the patient pathway. The patient's concerns related to the complex presentation of the disease and the access to the service.

The PWG consider that the commissioning arrangements will identify specialist centres more effectively and those centres will either have to provide the range of services required or evidence that they have networked care arrangements to enable appropriate patient access to the range of services they need. The policy will be clarified in this regard.

Prescribing will no longer be available through primary care; it is expected that this drug will be available through homecare delivery and the policy will be clarified in this regard.

4.5 Impact on equality and health inequalities

Respondents agreed that this policy would have a very positive impact on equality and health inequalities as the policy proposes increasing access to this treatment.

One respondent seemed unclear that the policy proposed routine commissioning but supported that principle.

4.6 Changes or additions

The majority of respondents did not think that policy needed changes or additions; the drug company wanted a reference to their yet to be launched product to be included.

The PWG did not think this would be appropriate; the drug is not yet marketed in England. The PWG noted that the EMA regard trientine tetrachloride as a hybrid of trientine dihydrochloride.

5. How have consultation responses been considered?

Responses have been carefully considered and noted in line with the following categories:

- Level 1: Incorporated into draft document immediately to improve accuracy or clarity
- Level 2: Issue has already been considered by the CRG in its development and therefore draft document requires no further change
- Level 3: Could result in a more substantial change, requiring further consideration by the CRG in its work programme and as part of the next iteration of the document
- Level 4: Falls outside of the scope of the specification and NHS England's direct commissioning responsibility

The **(*section redacted for publication*)** considers each response received to the consultation questions, and categorises the impact on the current policy document.

Of the 5 individual question responses received

- Two were categorised as level 1, where minor amendments have been incorporated into the policy document to improve accuracy or clarity.
- The remaining three responses received have been acknowledged but do not impact on the policy.

6. Has anything been changed in the policy as a result of the consultation?

The requirement to clarify that specialist centres must provide all the range of specialists required or be able to enable patient access through a networked care model has been included.

The policy will be amended to include the option of home care for drug delivery.

7. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposal?

There are no remaining concerns outstanding.