European-wide study confirms benefits of D-penicillamine and trientine for Wilson disease

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Results from the first ever European-wide retrospective analysis presented today at the International Liver CongressTM have shown both D-penicillamine and trientine continue to be effective treatments, providing positive survival rates in patients with Wilson disease free from a liver transplant.

Wilson disease is a rare genetic storage disorder in which copper is not excreted by the body effectively, leading to excess copper build up, liver failure and damage to the brain (neurological problems). Worldwide the condition, affects approximately one in 30,000 people. If not treated, Wilson disease can be chronically debilitating and life threatening. While current guidelines recommend the use of chelating agents (D-penicillamine, trientine) as first line therapy of symptomatic patients, optimal treatment regimens are yet to be established.

In this retrospective multicentre cohort study the long-term outcomes of treatment with D-penicillamine and trientine were reviewed in 347 patients with the condition. Changes of medication were common in both groups and resulted in a total of 467 analysed treatments (D-penicillamine, n=326, trientine n=141). Following an average follow-up of 16.5 years, both treatments provided a similar and excellent survival rate and prevented disease progression with the need for liver transplantation in over 98% of patients.

Daniele Prati, EASL's Scientific Committee Member and Press Committee Chairman commented: "Not only does this study show that both treatments are effective options for patients with this rare and often debilitating condition, but it is also encouraging to see how European countries can work together and exchange data which is extremely valuable to guide best practice and evaluate existing treatment regimens. Research on rare liver diseases presents remarkable interest."

"To date, a very limited number of drugs for these conditions are marketed, leaving the majority of rare diseases without any effective treatment. Research into rare diseases and possible cures needs to be strongly encouraged."

Follow-up at 48 months showed that liver deterioration occurred in only 8 out of 515 treatments (D-penicillamine n=4, trientine n=4) while neurological deterioration was less frequent in the D-penicillamine group (7/326 treatments) compared to trientine (12/141, $p \leq 0.05$). Improvements in liver function of symptomatic patients was similar between groups when data were stratified between first and second line treatments, with improvement rates during first line therapy in symptomatic patients of 90.7% for D-penicillamine and 92.6% for trientine.

More information: References

1. Weiss H.K et al, Efficacy and Safety of D-penicillamine and Trientine for the Treatment of Wilson Disease. Presented at the International Liver Congress 2011

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3. Wilson Disease Association. www.wilsondisease.org/about-wilsondisease.php . Accessed March 2011

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