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Wilson's disease: Prospective developments towards new therapies

Ranucci G *et al.* New perspectives on Wilson's disease

Giusy Ranucci, Roman Polishchuck, Raffaele Iorio

Abstract

Wilson's disease (WD) is an autosomal recessive disorder of copper metabolism, caused by mutations in the *ATP7B* gene. A clear demand for novel WD treatment strategies has emerged. Although therapies using zinc salts and copper chelators can effectively cure WD, these drugs exhibit limitations in a substantial pool of WD patients who develop intolerance and/or severe side effects. Several lines of research have indicated intriguing potential for novel strategies and targets for development of new therapies. Here, we

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against the somewhat different grading system used in the AASLD guidelines ... development of Wilson's disease is due to the accumulation of copper in affected medical therapy is rarely effective in patients presenting with acute liver failure Wilson's disease. A prospective study on serum ceruloplasmin as a screen-

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