Title: **Neuro-protective and cell treatments in patients with Wilson's Disease: Chinese experience**

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Abstract Congenital disorders of copper metabolism are associated with neurological/hepatically dysfunction including Wilson's disease (WD). WD is a autosomal recessive disorder caused by mutations in the ATP7B gene resulting in the inability of the hepatocytes to remove excess copper. Gradual copper accumulation causes damage to liver, brain and other organs manifesting in liver disease, neurological and psychiatric symptoms. Also scond copper-neurometaboic disorder: Menkes disease charaterized with mutated ATP7A gene, is ralated with abnormally neuroal transmission and synaptogenesis. There is over 30 000 WD inpatients in China University hospitals (Hefei , Shanghai, and Guangzhou cities), Most from Chinese, some Caucasians from USA and Europe and South Asia, and have been treated with better outcome approximately 80%. Besdies the anticopper agents (such as DMPS, DMSA, et al), together applied were Neuro-protective drugs: Butylphthalide ,Coenzyme Q, Butylphthalide, Edaravone and Vit E... Gene therapy and stem cell therapy also have been tried for pre-clinical or clinical trials. We, with Some other research groups, did successfully do the five stem-cell lines of WD. Induced pluripotent stem cell (iPSC) line, THSJTUi001-A, was generated from a 26-year-old Chinese male patient with Wilson's disease carrying a homozygous Arg778Leu mutation in ATP7B gene, using non-integrated episomal reprogramming vectors. This cell line had normal karyotype, expressed pluripotency markers and could differentiate into the three germ layers in vivo. The authors may plan to target at the some points of brain or liver.