Wilson disease treatment - we need more options

K. H. Weiss
Internal Medicine IV, University Hospital Heidelberg, Germany

Wilson Disease is a rare genetic disorder of impaired copper transport and excretion, caused by loss of function of the ATP7B copper-transporter. Wilson Disease is clinically primarily characterized by varying degrees of liver, neurological and psychiatric manifestations related to damage toxicity of excess copper causes to the liver and brain. Treatment of Wilson Disease remains largely unchanged since the introduction of chelators and zinc. With the current standard treatments, improving symptoms can take several years and patients with neurological symptoms show a poorer response to therapy than hepatic patients. Thus considerable unmet medical needs still exist, what will be discussed in the presentation. These unmet needs include more effective copper control, improving symptoms and associated disabilities, reducing the risk of neurological worsening after treatment initiation and simplification of dosing regimen for improved compliance.