Vivet THERAPEUTICS

A GUIDE TO WILSON'S DISEASE

This leaflet will provide you with information about **Wilson's disease**, including **symptoms, causes** and **current treatments**

What is Wilson's disease?

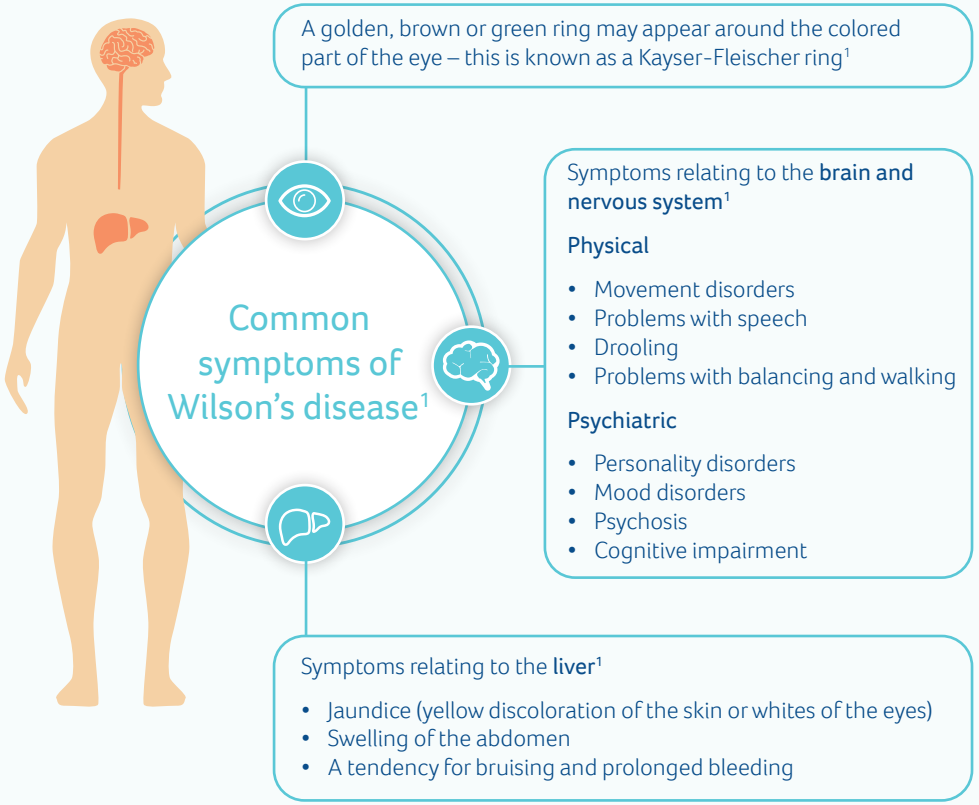
Wilson's disease is a rare genetic disorder that affects approximately 1 in 30,000 people worldwide.[1] It causes excess copper to build up in the body, particularly in the liver and brain.[2]

Small amounts of copper, an essential nutrient, are required by our body to perform many important functions.[1,3] The body cannot make copper, but acquires it from foods in our diet, such as potatoes, nuts, seeds, chocolate, meat and shellfish.[1] While it is essential for health, too much copper can damage our organs, so our bodies carefully control the amount that is removed ('excreted').[2] In Wilson's disease, the copper is insufficiently excreted and builds up in the body.[2]

If Wilson's disease is not diagnosed early and appropriately treated, the build-up of copper will cause serious damage to organs and may result in death.[1,2] This means that early diagnosis and prompt treatment of Wilson's disease is very important.[1]

What are the symptoms of Wilson's disease?

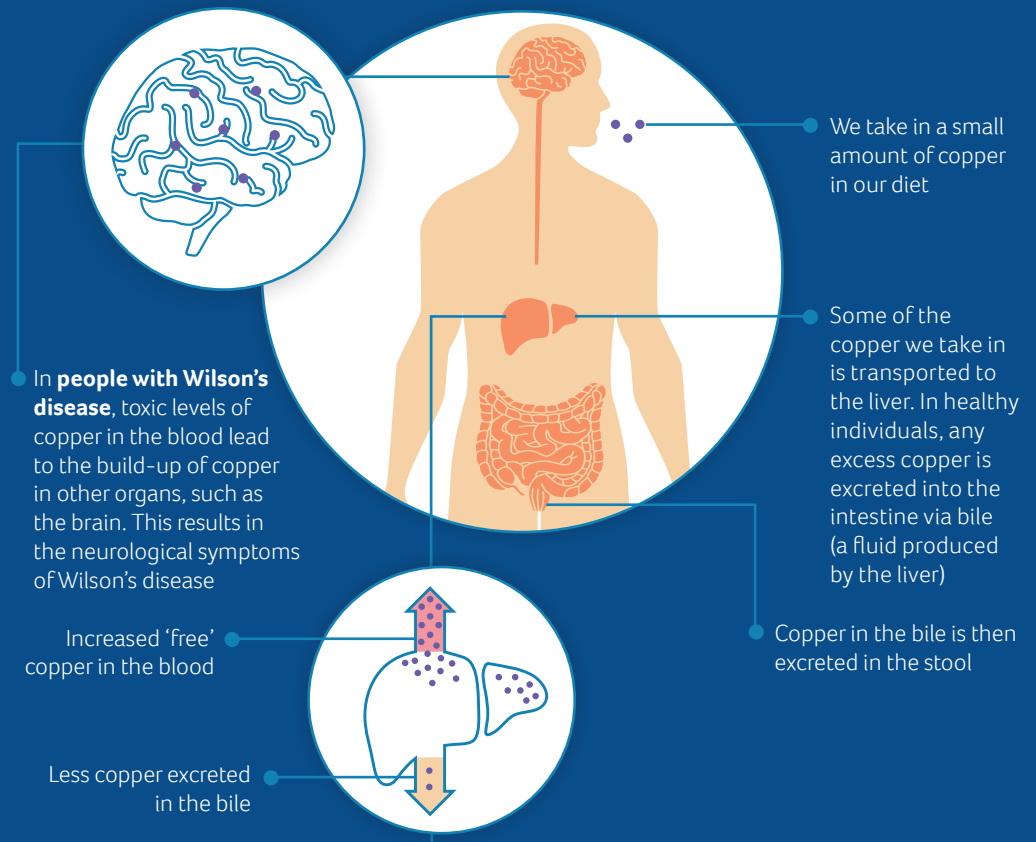
Symptoms may appear at any time, but usually between the ages of 5 and 35 years.[4] They also vary widely between people with Wilson's disease.[1]



What is the cause of Wilson's disease?

Wilson's disease is an inherited genetic disease. It arises when a faulty gene is passed to a child from both parents.[1] A gene is a segment of our DNA that has the instructions for making a specific protein in the body. The faulty gene in Wilson's disease is responsible for making a protein called ATP7B, which helps to remove copper from the liver.[1] This gene is abnormal in Wilson's disease, causing ATP7B not to function properly.[1] This means the body cannot properly control copper levels, resulting in a build-up of copper in the organs.[1]

How does excess copper build up in the body and cause symptoms in Wilson's disease?[1,5]



How is Wilson's disease treated?

Currently, there is no cure for Wilson's disease, but there are several oral medications that can help to improve symptoms and prevent disease progression. The available medications do this by reducing the amount of copper that has built up in the body and keeping it at acceptable levels.[2] These medications need to be taken throughout a patient's life. A low-copper diet can also help to limit the amount of copper in the body.[1]

There are two main types of medication:[1]

- Copper chelators, which bind to the copper that has built up in the body so that it can be eliminated through the urine
- Zinc salts, which prevent the gut from absorbing copper from the diet

For patients with severe liver damage due to Wilson's disease, who do not respond sufficiently to the available medications, a liver transplant may be considered.[1]

People with Wilson's disease should also be careful to limit the amount of copper in their diet. Foods such as nuts, seeds, chocolate, organ meat (liver in particular) and shellfish are high in copper content.[1]

i Vivet Therapeutics is conducting a clinical trial to evaluate the effects of a new treatment for Wilson's disease. If you would like to know more about the trial, talk to your treating specialist

For further information on Wilson's disease:

Wilson Disease Association (www.wilsonsdisease.org)
National Organization for Rare Disorders (https://rarediseases.org/organizations/wilson-disease-association/)
EuroWilson (http://www.eurowilson.org)

References:

1. Członkowska A *et al.* Wilson disease. *Nat Rev Dis Primers* 2018;4:21.
2. Wilson's Disease Association (www.wilsonsdisease.org). Last accessed June 2020.
3. Uauy R *et al.* Essentiality of copper in humans. *Am J Clin Nutr* 1998;67:952S–9S.
4. European Association for the Study of the Liver (EASL). EASL Clinical Practice Guidelines: Wilson's disease. *J Hepatol* 2012;56:671–85.
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