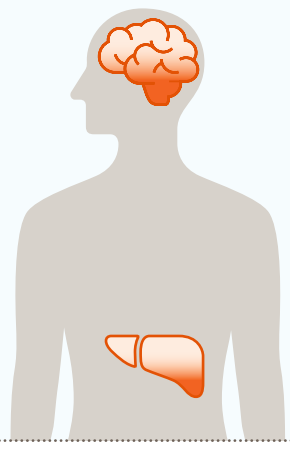
Wilson Disease

AstraZeneca Rare Disease

What is Wilson disease?

Wilson disease is a **rare and progressive genetic (inherited) condition** in which the body's **pathway for removing excess copper is compromised**.[1]

Over time, that results in the **build-up of toxic copper levels** in the liver, brain and other organs leading to damage that greatly impacts a patient's life.[1]



Diagnosed prevalence is

~5K **~5K** **~2.5K**

but prevalence is believed to be higher.[2]

29 Cu COPPER

Copper is an important nutrient that is **not produced by the body**, but **absorbed from a person's diet** and only required in small amounts.[3]

Patients can develop a wide range of symptoms, including **liver disease** and/or **psychiatric or neurological symptoms**, such as:

- **personality changes** (such as depression, anxiety and phobias)
- **tremors**
- **difficulty walking**
- **difficulty swallowing**
- **difficulty talking**
- **changes to the cornea**

In some cases, **the damage and loss of function may be irreversible**.[1,4,5]

How is Wilson disease diagnosed?

blood tests · liver biopsy · eye exam · urine test · genetic testing

Diagnosis typically requires a combination of **5+ tests**, scoring system of **7 different signs and symptoms** and/or invasive procedures like a liver biopsy.[5]

Although the disease is present at birth, the average age of diagnosis is **5-35 years.**[4,5]

People living with Wilson disease frequently face two to three years of misdiagnoses.[6]

2–3 years

Early, improved diagnosis is key to enable earlier treatment and **help reduce the risk of worsening organ damage**.[5,7]

What are the current treatment needs?

Existing standard-of-care treatments either remove copper from the blood or limit copper absorption in the digestive system.[4,5] However, even after treatment is initiated, some patients experience **worsening of disease, especially of neurologic symptoms**.[4,5]

These treatments can be **difficult to tolerate** and have **challenging dosing schedules.**[8,9]

What treatment approach is being studied by Alexion?

Alexion is advancing the **first potential new innovation in treating Wilson disease in more than 30 years.**[10]

ALXN1840 is designed to be the **first targeted de-coppering therapy** that **selectively and tightly binds to and removes copper** from the body's tissues and blood.

This once-daily, oral medicine has the **potential to change the disease trajectory** and what it means to live with Wilson disease.[11]

ALXN1840 is not approved for the treatment of Wilson disease. The safety and efficacy of ALXN1840 for the treatment of Wilson disease is currently being studied.

References:

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