

# Gene Therapy for Wilson Disease

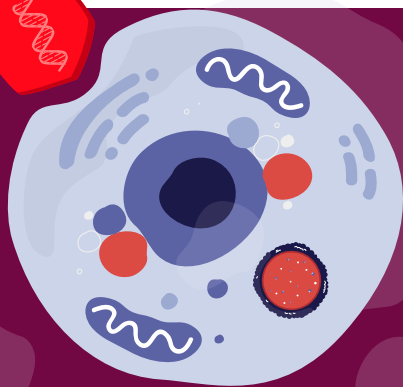
## The *ATP7B* Gene and Wilson Disease

Wilson Disease is caused by mutations or changes to the *ATP7B* gene. This gene instructs cells to produce a transporter protein which moves excess copper out of the liver. With a faulty *ATP7B* gene, the transporter protein activity level is low or absent, causing excess copper to accumulate in the liver and become toxic.



*ATP7B* Gene

Viral Vector

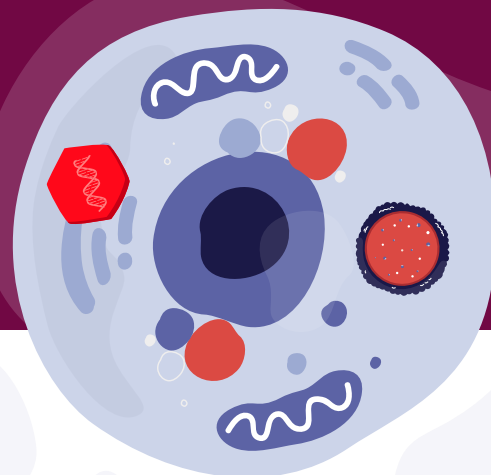


## Delivering the Vector

Scientists know that viruses are good at getting into cells, so they have learned how to safely use this ability to get into cells as a vector—or carrier—to deliver the gene. But don't worry, the viral genes are removed so that only therapeutic genes are delivered.

## How Gene Therapy Can Help

Gene therapy aims to be a one-time treatment that delivers a working *ATP7B* gene into cells that produce working transporter proteins. The goal is to restore proper liver function to remove excess copper and prevent further build up. Viral vectors are used to deliver the gene to cells, which is given through an intravenous infusion to target liver cells.



## The Role of Clinical Trials

This investigative therapy for Wilson Disease is currently being researched in clinical trials. Clinical trials are part of a required research process to determine if a treatment is safe and effective. People need to meet certain eligibility criteria to participate in clinical trials, which can include age of symptom onset and history of medical treatment.

