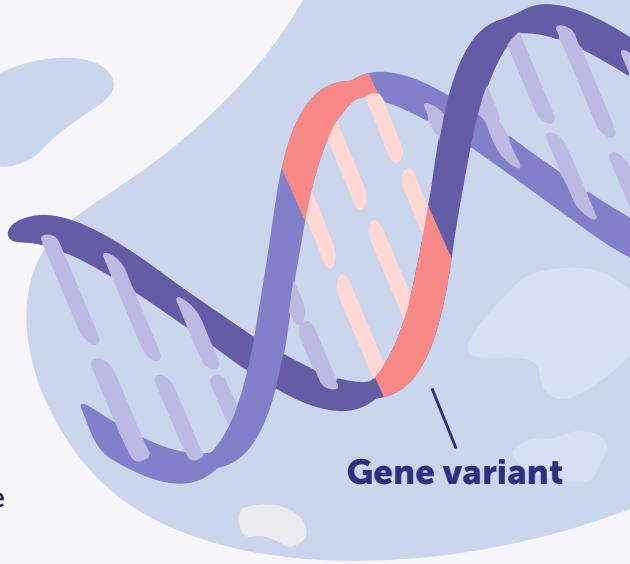
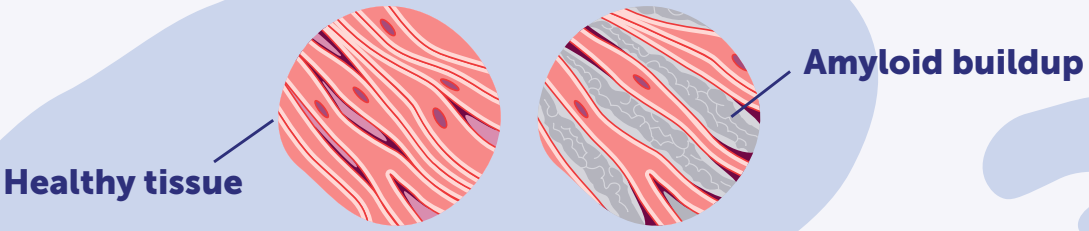
Gene Therapy for ATTR

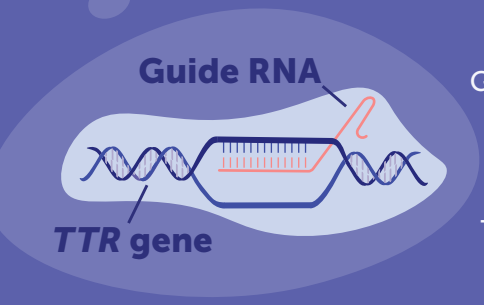
The Effect of Amyloid Deposits

There are more than one hundred genetic variants that can cause transthyretin amyloidosis (ATTR). Some ATTR conditions are caused by changes to the *TTR* gene, resulting in cells not producing proper TTR proteins.

This leads to a buildup of misfolded proteins, called amyloids, where the proteins clump together in organs, nerves, or tissues causing concerns throughout the body.







Gene Editing Approach

Gene editing aims to correct pieces of DNA by changing or deleting information within the gene. CRISPR Cas9 is a gene editing technique made of two components, a piece of modified RNA called guide RNA and a protein.

The guide RNA locates the *TTR* gene, and the protein makes the edit to remove the *TTR* gene entirely from the DNA sequence. This approach is done *in-vivo,* meaning it is delivered into the body through an injection. A viral vector carries the genetic material into the cells, where the CRISPR Cas9 instructs the liver cells to stop making the TTR protein that was causing the harmful amyloid deposits.

Considering Treatment Options

Gene editing and other gene therapy approaches are being researched in clinical trials to determine if they are safe and effective.

There is an FDA-approved gene therapy that uses gene silencers to target a gene's messenger RNA to prevent production of unwanted proteins.

It is important to discuss any potential treatments with a trusted healthcare provider.

American Society of Gene + Cell Therapy

Visit patienteducation.asgct.org for more information