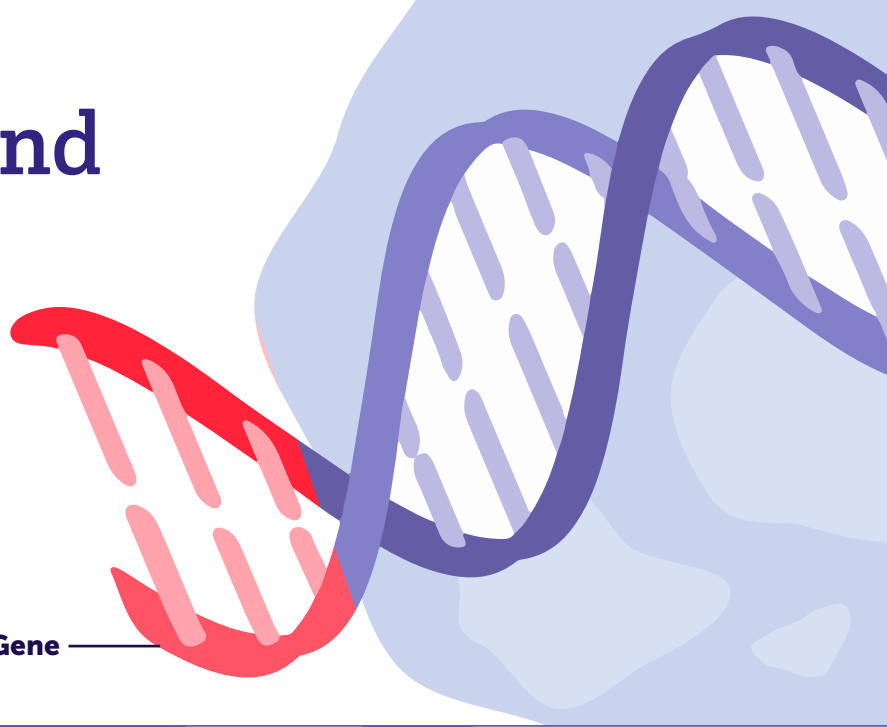


Batten Disease and Gene Therapy

The Cause

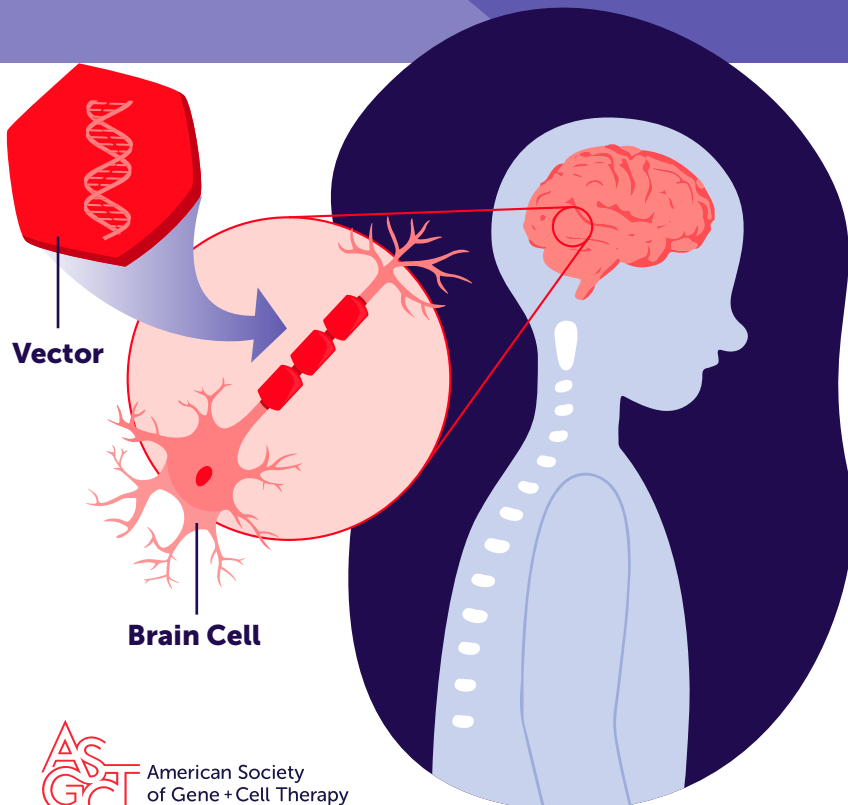
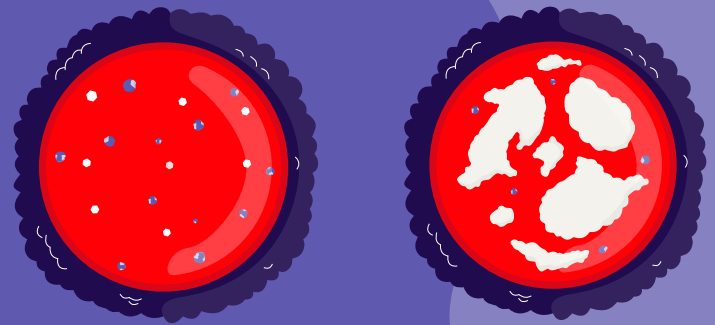
This group of rare, inherited disorders affects the nervous system, resulting in a rapid decline of vision, movement, and thinking abilities. Each 1 of 13 known forms is linked to a mutation in a certain ceroid lipofuscinosis (CLN) gene.

Faulty Gene



Limiting Lysosomes

The faulty genes linked to Batten disease cause deficiencies in key enzymes that limit the lysosomes' ability to function properly. Lysosomes are a part of the cell known as the "recycling center" since they break down and remove waste. Without proper function, waste builds up and causes progressive damage to the brain and body.



Goal of Gene Therapy

Gene therapy aims to slow or even stop the progression of disease by delivering a working gene into cells of the brain and spinal cord. A vector, which is derived from a virus, is used to deliver the genetic material because it is good at getting into cells. But, the viral genes are removed, so only the therapeutic genes are delivered. The new genes are able to instruct cells to produce the correct enzymes and restore lysosomal function. As each form of Batten disease is unique, each one needs its own gene therapy targeting the specific gene mutation.