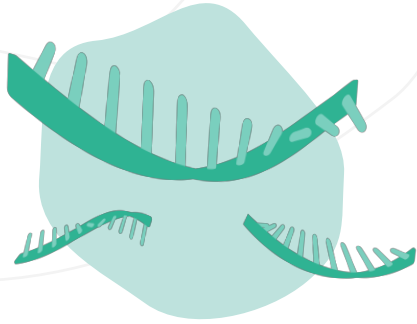
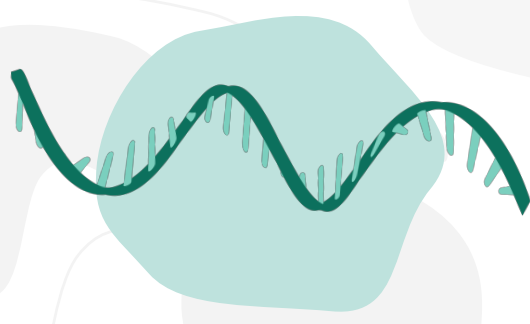


Huntington's Disease targeted by MicroRNAs and ASOs



Meet the MicroRNAs

MicroRNAs are molecules that act by silencing RNAs instructed in cells to create proteins or enzymes.



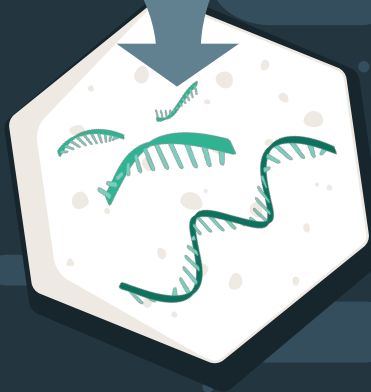
Making Sense of Antisense

Antisense oligonucleotides, 'ASOs', are DNA -or RNA-like molecules that can be delivered into cells to bind to genes and change how they produce proteins or enzymes.

Role in Gene Therapy

MicroRNAs can be delivered into cells using vectors, which are usually derived from viruses, but modified so all viral genes are removed. The vector transports this genetic material into cells to block or slow how the faulty HTT gene produces toxic proteins.

Antisense therapies employ the use of ASOs that are delivered into the diseased cells to block or alter how cells express genes. There, they can modify how target RNAs are processed and expressed to control the faulty HTT gene.



Different with the Same Goal

Although both the microRNAs and ASOs are designed in a specialized lab, therapeutic microRNAs are based on natural genes found in humans. Also, antisense therapies typically requires multiple doses. Both approaches aim to limit the toxic protein in the brain resulting in slower breakdown of neurons that would lead to better managed symptoms of Huntington's disease.