



## **Non-Confidential Technology Disclosure**

**Titles:** RNAi treatment for trinucleotide repeat diseases

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**Description:** Trinucleotide repeat diseases, including Huntington's disease, are associated with neurodegeneration in the brain and/ or spinal cord. The common mutation among this group of diseases is an increase in a particular trinucleotide repeat that produces a dominant, gain-of-function protein. While the exact pathogenic pathways are yet to be determined, disease is thought to be caused by aberrant protein interactions. This invention describes treatment of trinucleotide repeat diseases, such as Huntington's disease, by the use of RNA interference (RNAi) to target and destroy the RNA that encodes the mutant proteins while allowing expression of the remaining, non-mutated allele.

**Application:** Small-interfering RNA (siRNA) can be used to specifically target allelic polymorphisms within a gene containing a dominant, gain-of-function mutation that is the hallmark of trinucleotide repeat disorders such as Huntington's disease.

**Advantage:** There is currently no effective treatment for the severe neurodegenerative trinucleotide repeat diseases such as Huntington's. The use of siRNA molecules to specifically target allelic polymorphisms, as opposed to the disease causing repeat expansion, is a novel method that may provide the first effective treatment for these trinucleotide repeat diseases. Allelic polymorphisms, including deletion mutations, insertion mutations, and point mutations, would be directly targeted to avoid complications arising from targeting the repeat region. Use of RNAi as a treatment would allow selective silencing of the dominant, gain-of-function mutant allele thereby eliminating the toxicity that causes the disease while retaining normal expression of the wild-type allele.

**Patent Status:** Patent pending

**Licensing Status:** Available to license

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