UTSouthwestern Medical Center

TITLE: Allele Selective Inhibition of Mutant C90RF72 Foci Expression by Duplex RNAs Targeting the Expanded Hexanucleotide
INVENTORS: David Corey.
TECHNOLOGY: Biologicals
UTSD: 2851

SUMMARY: C9ORF72 is a known gene with currently undetermined function. It has been linked to familial ALS. The mutant allele contains and expanded hexanucleotide repeat (GGGGCC) that forms foci within cells that likely cause the disease. This entire region is composed of G's and C's and form many stable base pairs making it very difficult to manipulate. This invention reveals that duplex RNAs can reverse foci formation. They target both sense and antisense transcripts at the C9ORF72 locus. Blocking both of them provides a useful therapeutic strategy. This technology is different from applying general antisense technique targeting GGGGCC repeats.

Please contact the Office for Technology Development for more details:

Phone: 214-648-1888
Email: TechnologyDevelopment@utsouthwestern.edu

Please reference UT Southwestern Case Number: **2851**