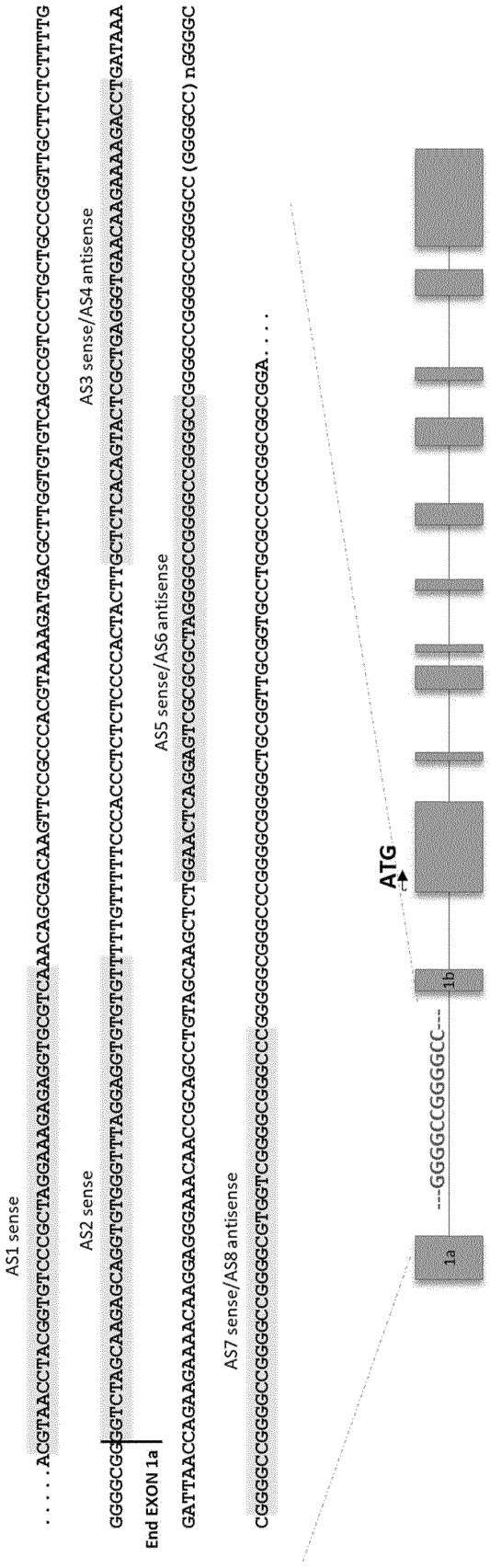

AS1 sense
.....ACGTAACCTACGGTGTCCCGCTAGGAAAGAGAGGTGCGTCAAACAGCGACAAGTTCCGCCCACGTAAAAGATGACGCTTGGTGTGTCAGCCGTCCCTGCTGCCCGGTTGCTTCTCTTTTG
AS2 sense
GGGGCGGGGTCTAGCAAGAGCAGGTGTGGTGTTTTTGTTTTTTGTTTTTTCCCACCCTCTCTCCCCACTACTTGCTCTCACAGTACTCGCTGAGGGTGAACAAGAAAAGACCTGATAAA
AS3 sense/AS4 antisense
End EXON 1a
AS5 sense/AS6 antisense
GATTAACCAGAAGAAAACAAGGAGGGAAACAACCGCAGCCTGTAGCAAGCTCTGGAACTCAGGAGTCGCGCGCTAGGGGCCGGGGCCGGGGCCGGGGCCGGGGCC (GGGGCC) nGGGGC
AS7 sense/AS8 antisense
CGGGGCCGGGGCCGGGGCCGGGGCGTGGTCGGGGCGGGCCCGGGGGCGGGGCTGCGGTTGCGGTGCCTGCGCCCGCGGCGGCGGA. . . .
ATG
---GGGGCCGGGGGCC---
1a
1b

FIGURE 1

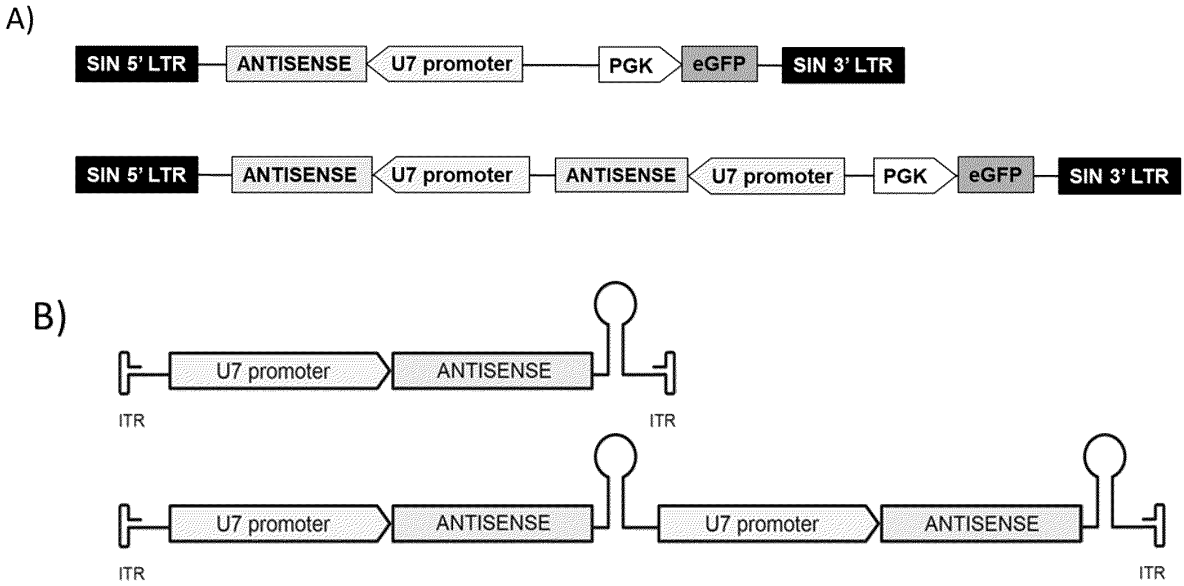


FIGURE 2

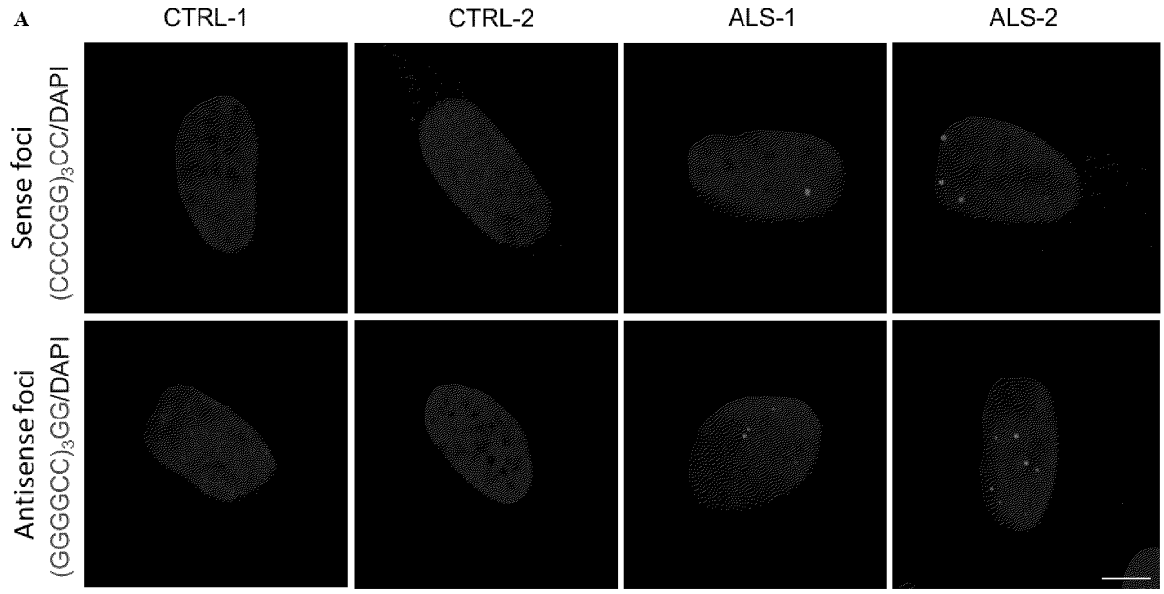
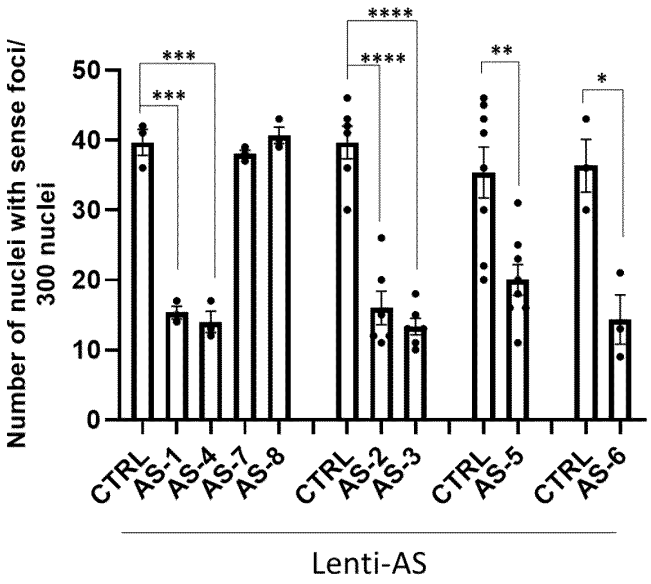


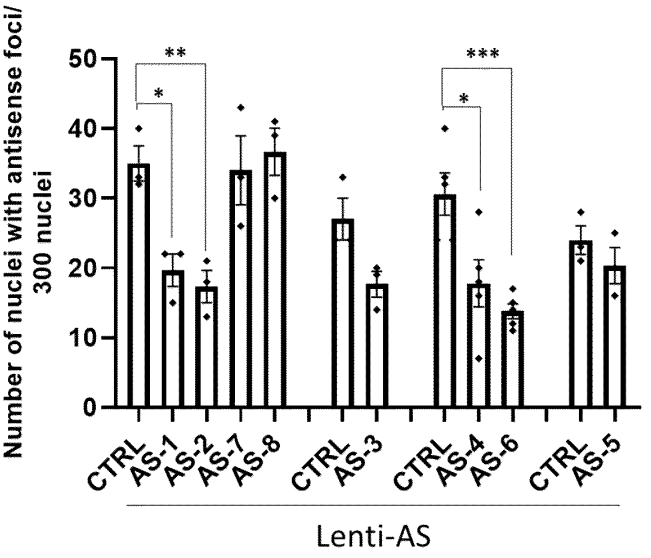
FIGURE 3

A)



	AS-1	AS-2	AS-3	AS-4	AS-5	AS-6	AS-7	AS-8
% of sense foci reduction	62%	59%	66%	64%	43%	61%	3%	0%

B)



	AS-1	AS-2	AS-3	AS-4	AS-5	AS-6	AS-7	AS-8
% of antisense foci reduction	44%	50%	35%	42%	15%	55%	3%	0%

FIGURE 4

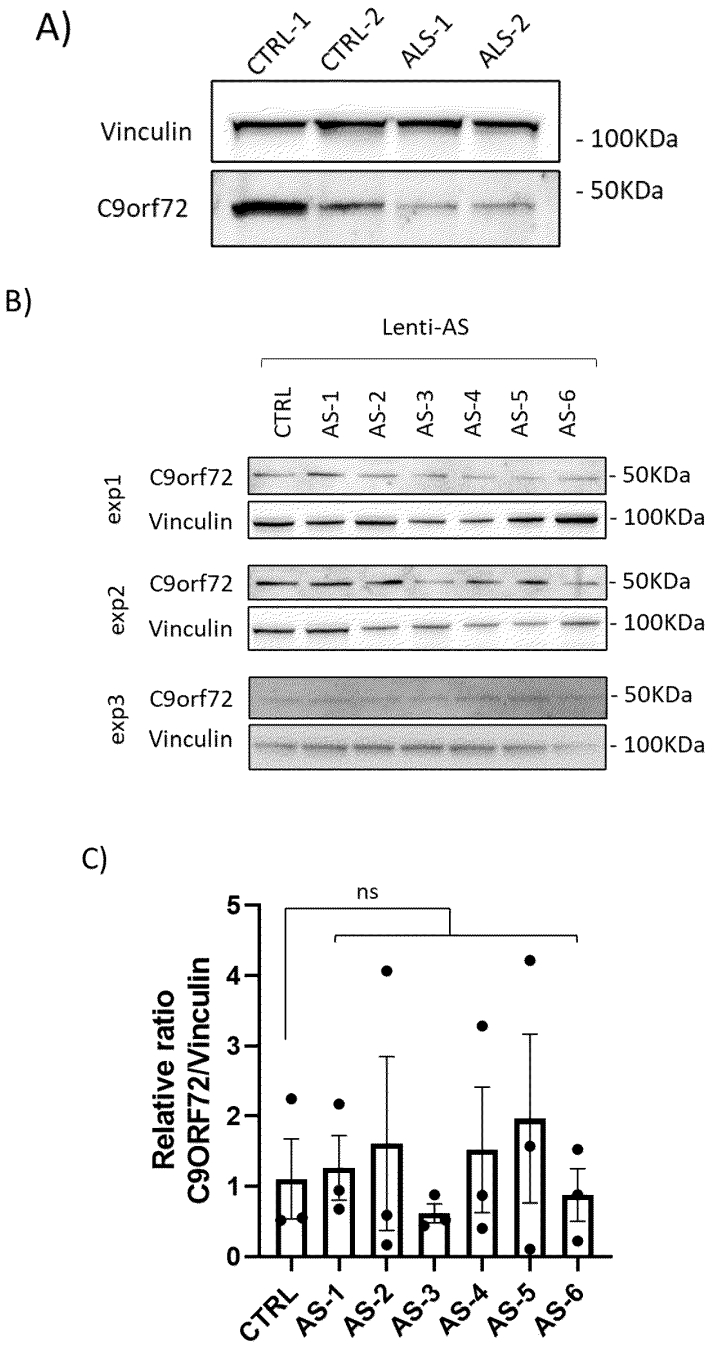
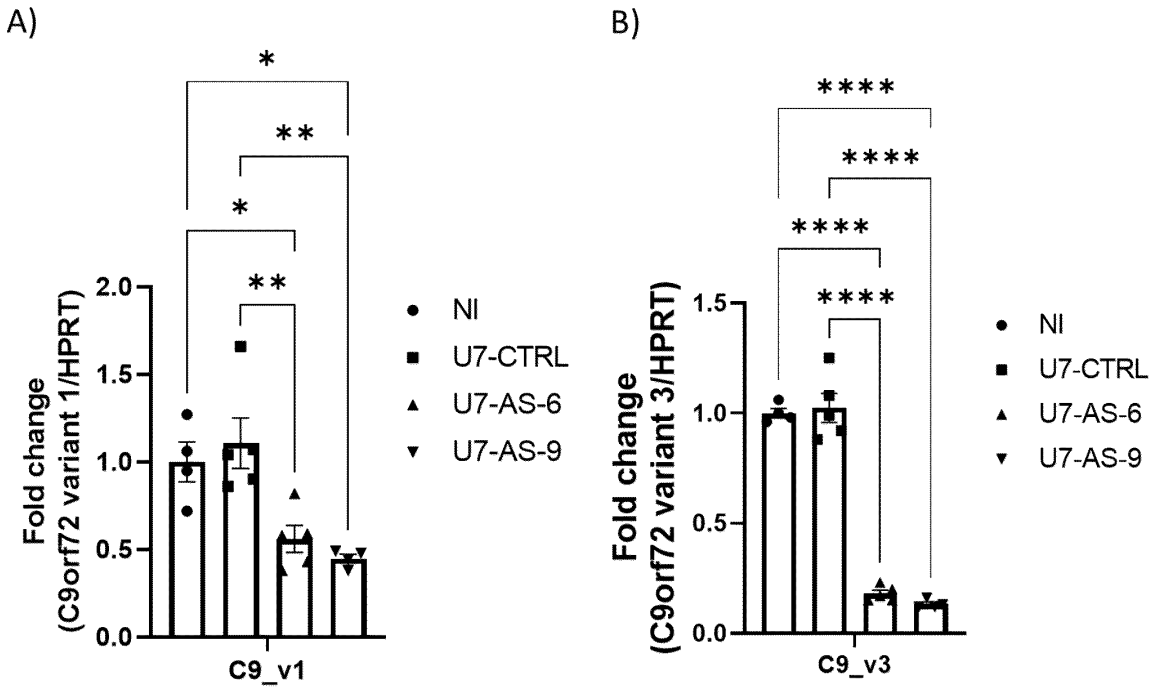

A)
CTRL-1
CTRL-2
ALS-1
ALS-2
Vinculin
- 100KDa
C9orf72
- 50KDa
B)
Lenti-AS
CTRL
AS-1
AS-2
AS-3
AS-4
AS-5
AS-6
exp1
C9orf72
- 50KDa
Vinculin
- 100KDa
exp2
C9orf72
- 50KDa
Vinculin
- 100KDa
exp3
C9orf72
- 50KDa
Vinculin
- 100KDa
C)
ns
Relative ratio C9ORF72/Vinculin
0
1
2
3
4
5
CTRL
AS-1
AS-2
AS-3
AS-4
AS-5
AS-6

FIGURE 5



	AS-6	AS-9
% of C9orf72 variant 1 reduction	44% vs NI 49% vs U7-CTRL	55% vs NI 59% vs U7-CTRL
% of C9orf72 variant 3 reduction	82% vs NI and vs U7-CTRL	87% vs NI and vs U7-CTRL

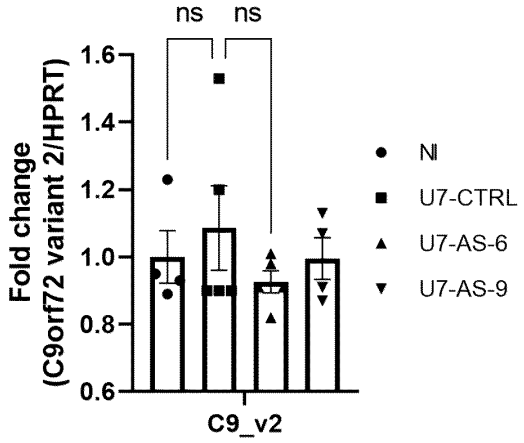


FIGURE 6