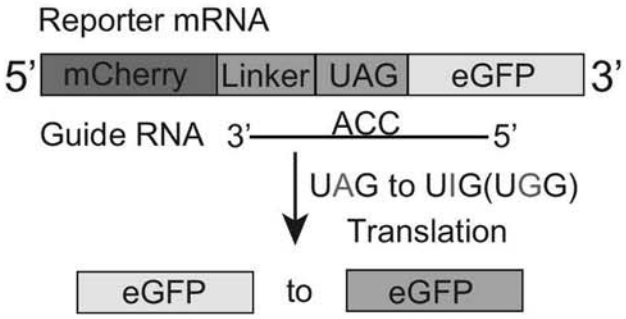


Figure.1A

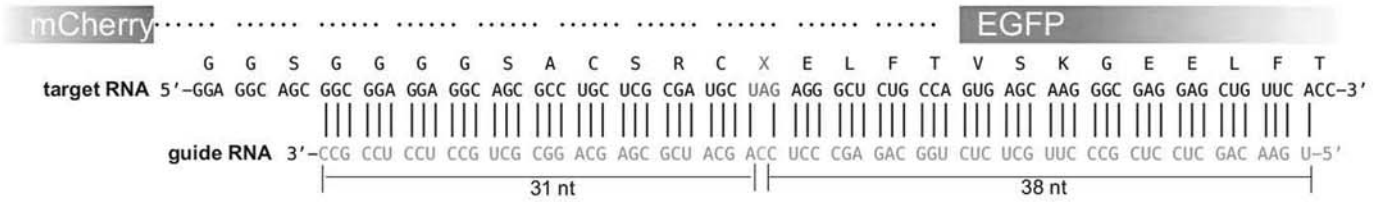


Figure.1B

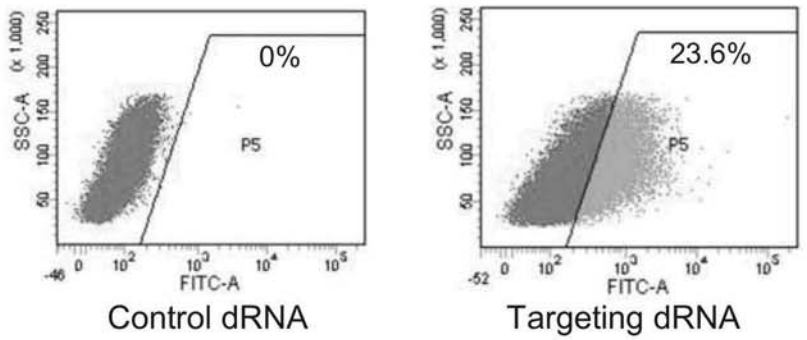


Figure.1C

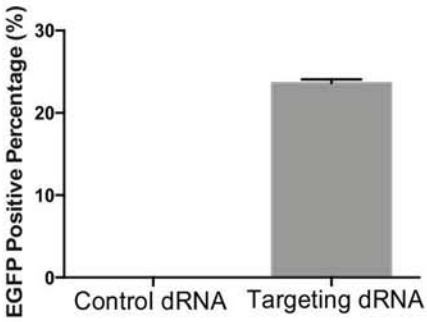


Figure.1D

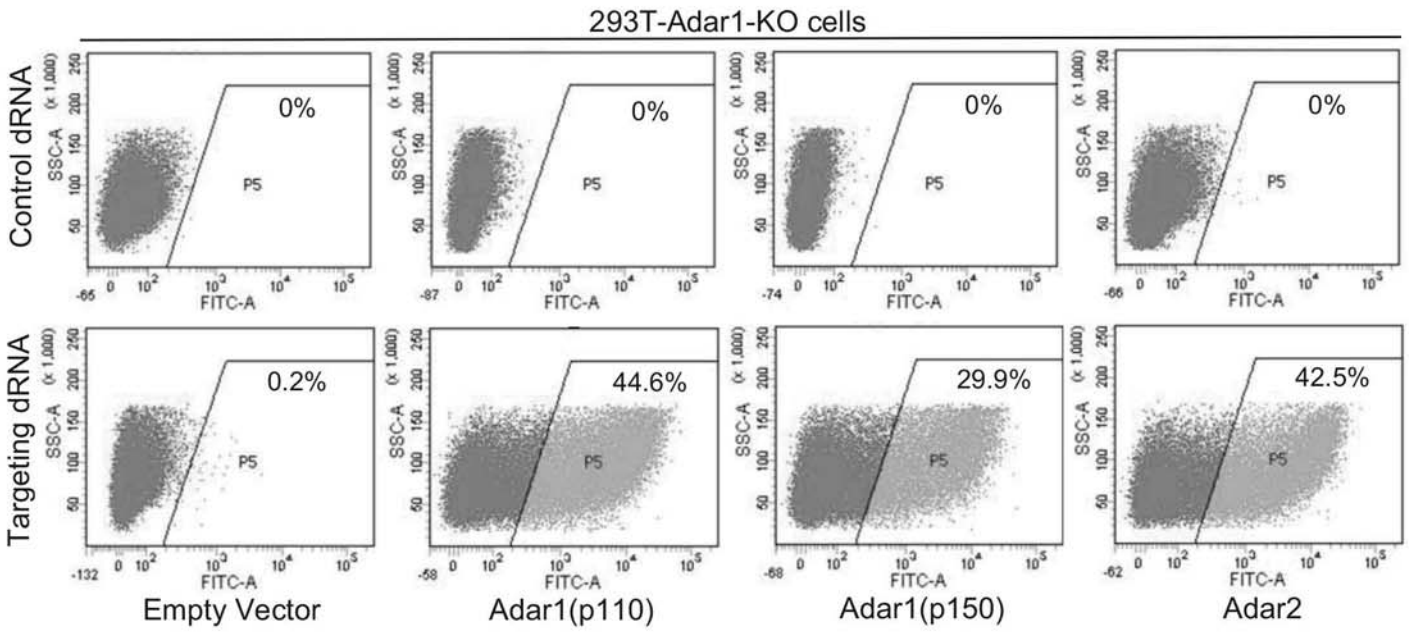


Figure.1E

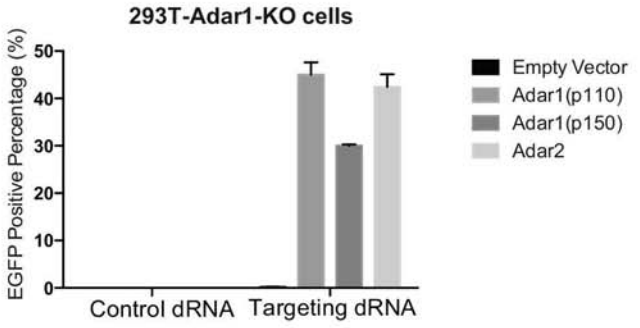


Figure.1F

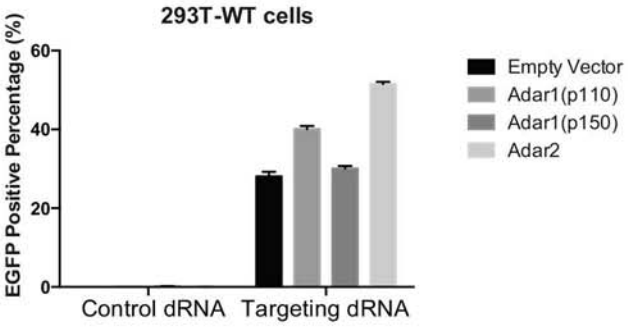


Figure.1G

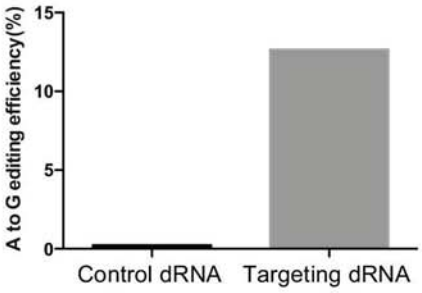


Figure.1H

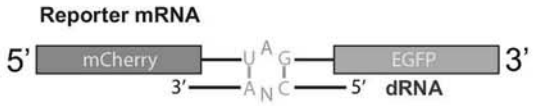


Figure.2A

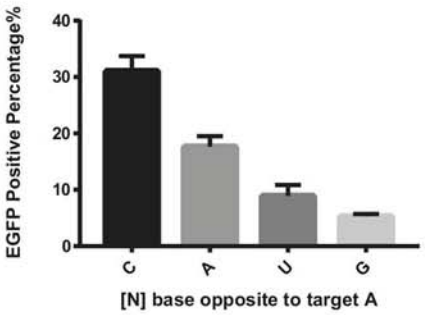


Figure.2B

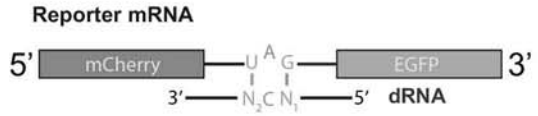


Figure.2C

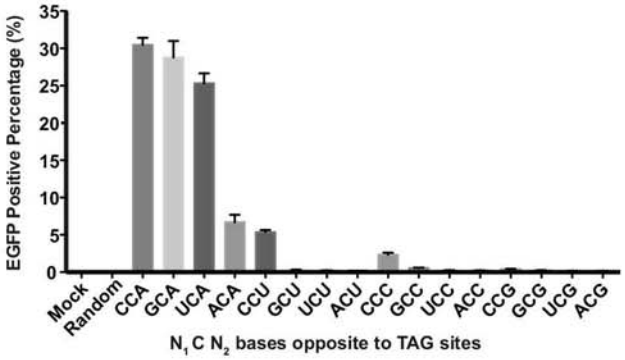


Figure.2D

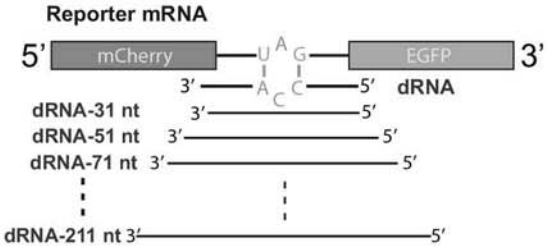


Figure.2E

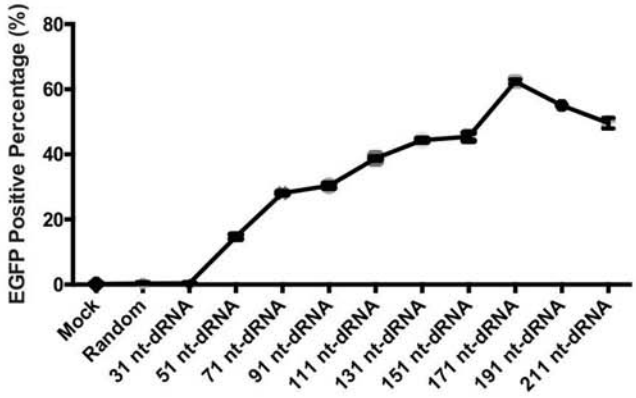


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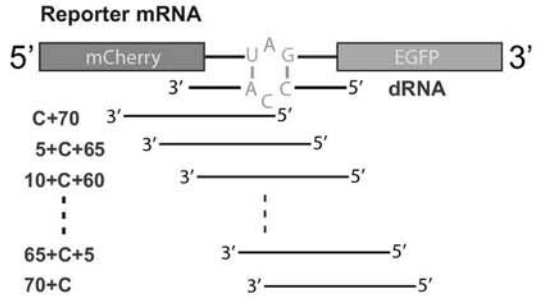


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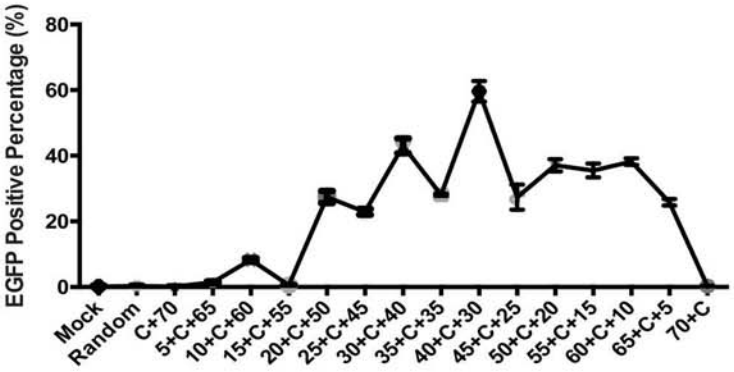


Figure.2H

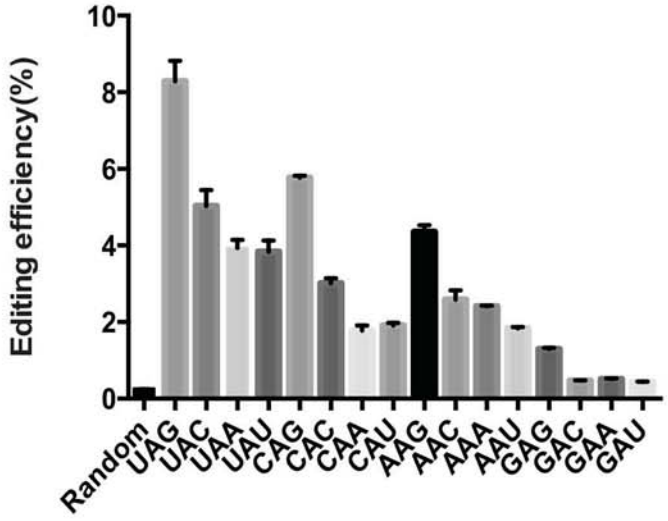


Figure.3A

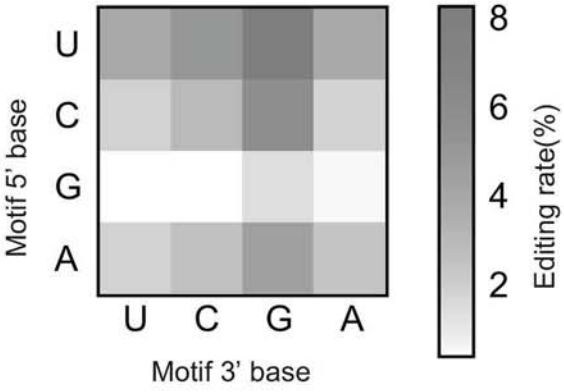


Figure.3B

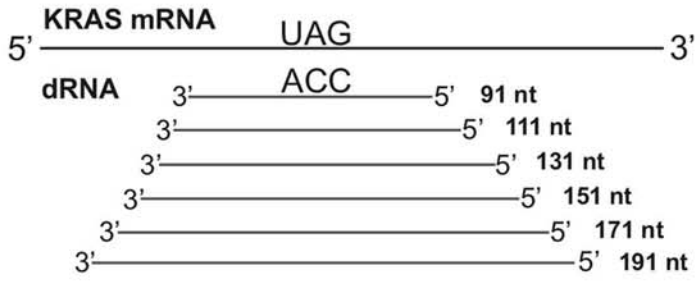


Figure.4A

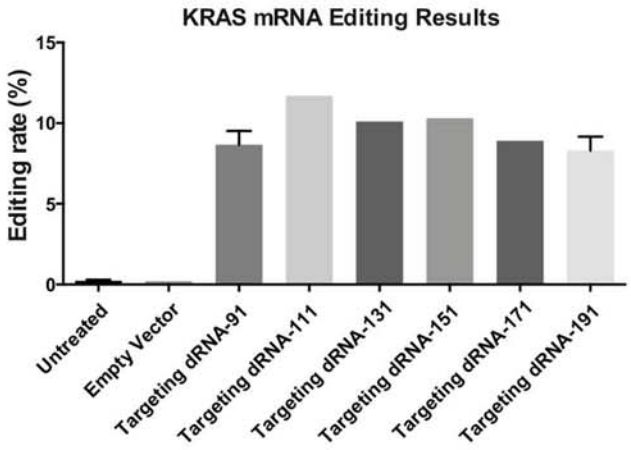


Figure.4B

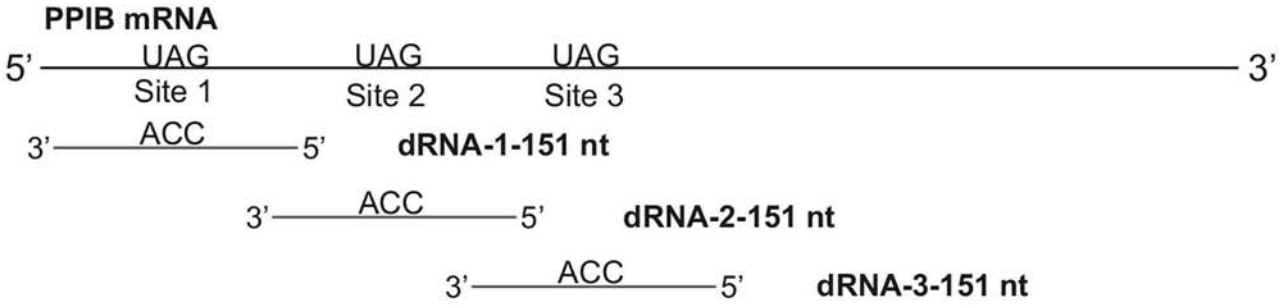


Figure.4C

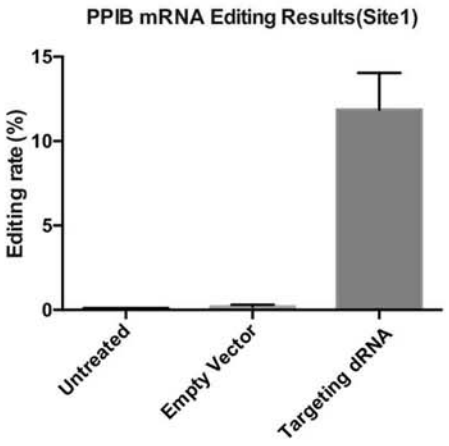


Figure.4D

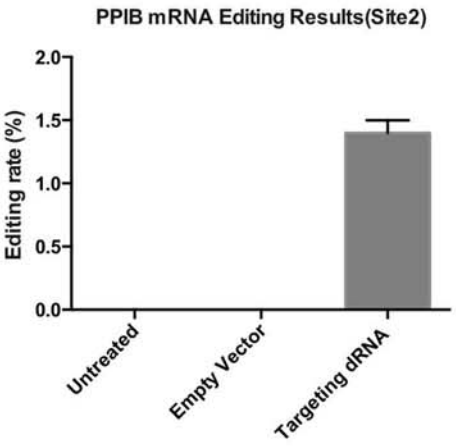


Figure.4E

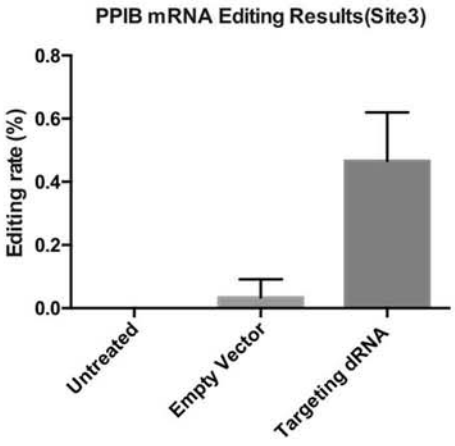


Figure.4F

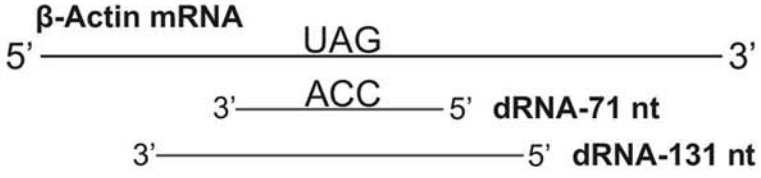


Figure.4G

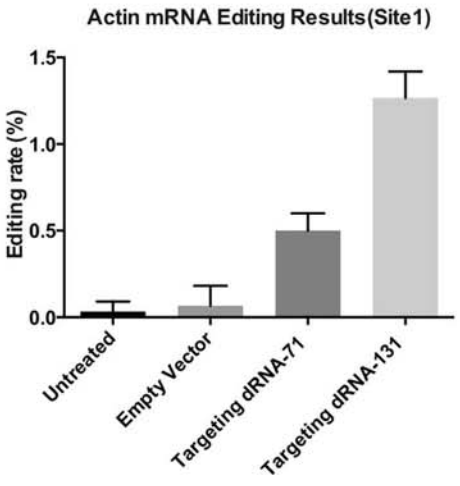


Figure.4H

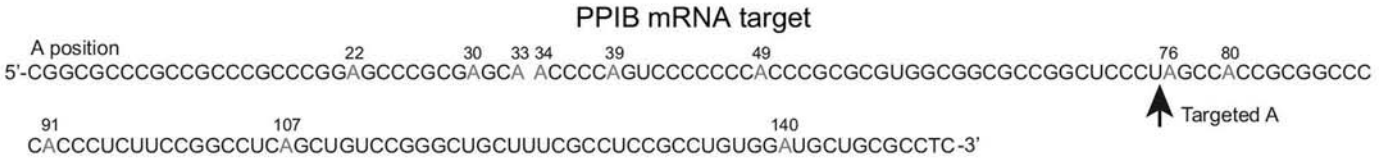


Figure.5A

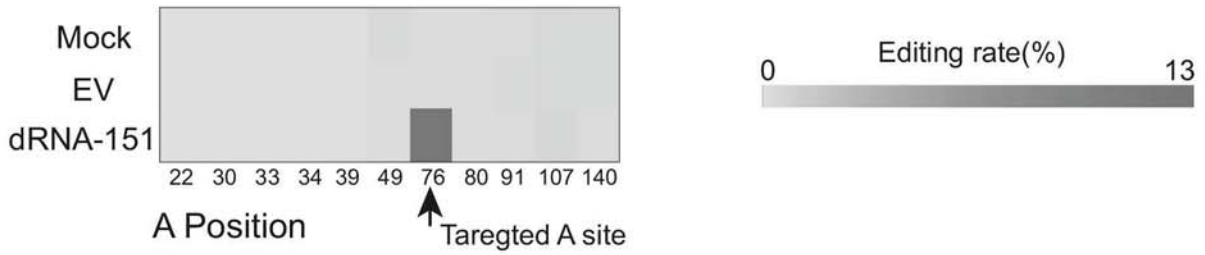


Figure.5B

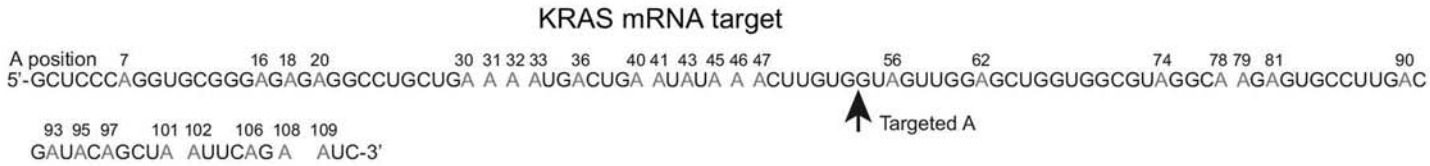


Figure.5C

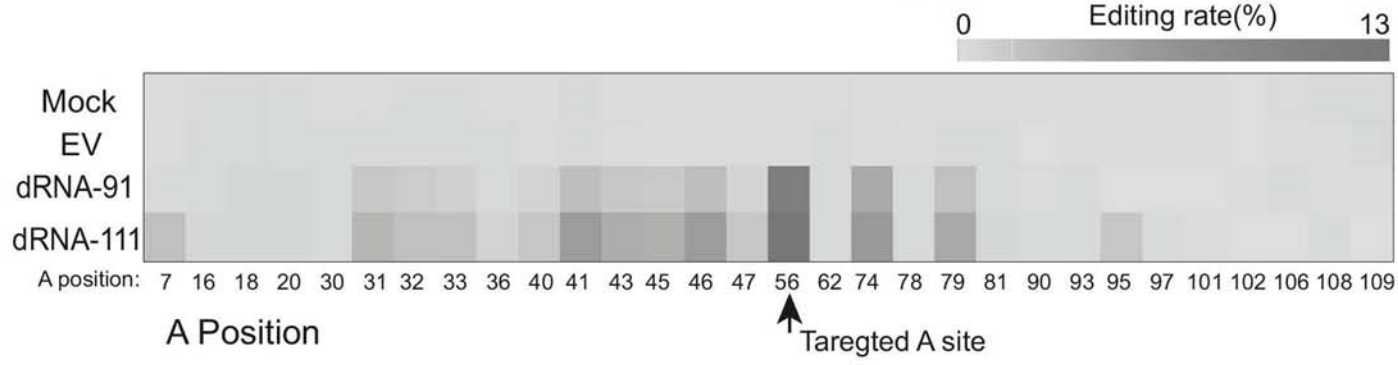


Figure.5D

A-G mismatch sites

dRNA-AG1: A_{41} , A_{46} , A_{74}

dRNA-AG2: A_{41} , A_{43} , A_{45} , A_{46} , A_{74} , A_{79}

dRNA-AG3: A_{31} , A_{32} , A_{33} , A_{41} , A_{43} , A_{45} , A_{46} , A_{47} , A_{74} , A_{79}

dRNA-AG4: A_{7} , A_{31} , A_{32} , A_{33} , A_{40} , A_{41} , A_{43} , A_{45} , A_{46} , A_{47} , A_{74} , A_{79} , A_{95}

Figure.5E

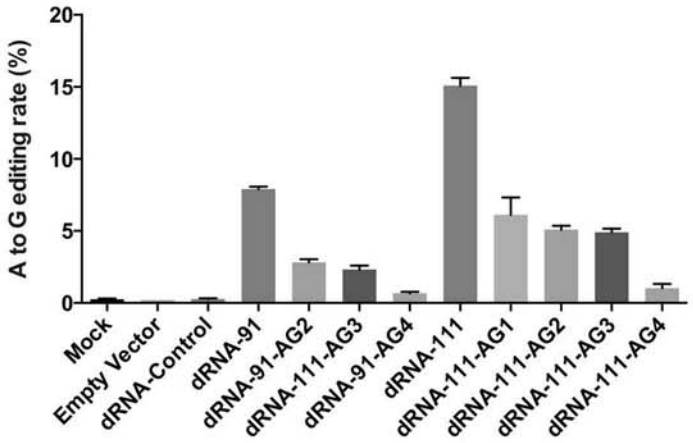


Figure.5F

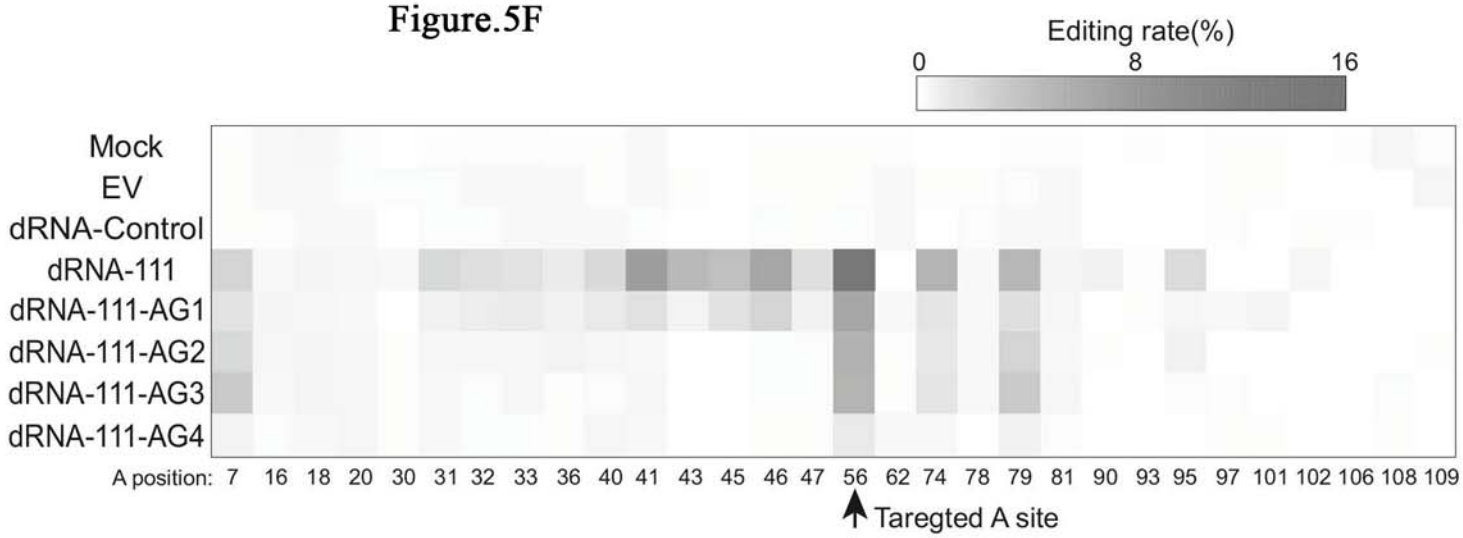


Figure.5G

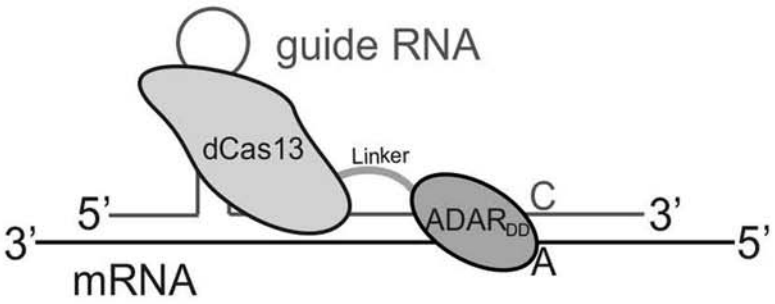


Figure.6A

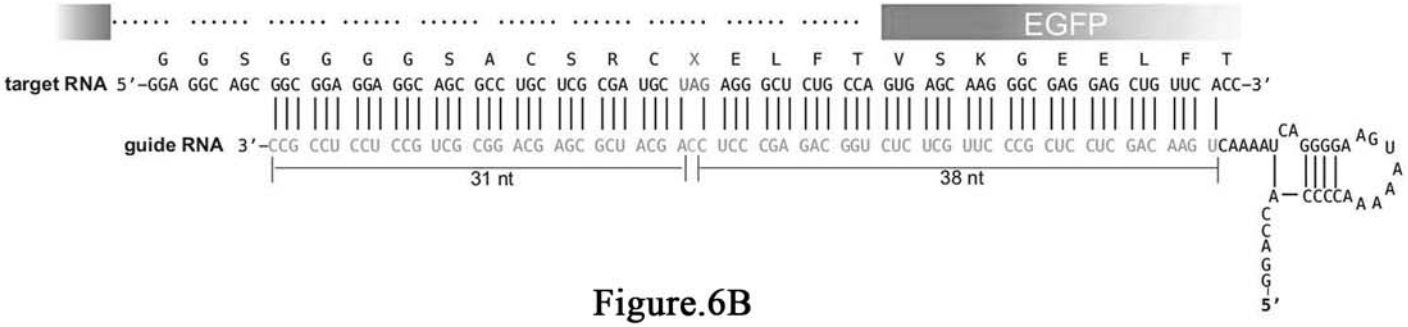


Figure.6B

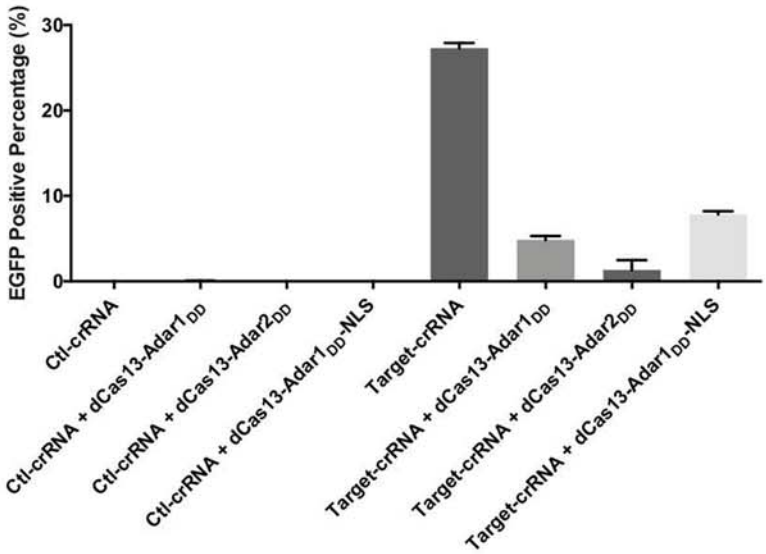


Figure.6C

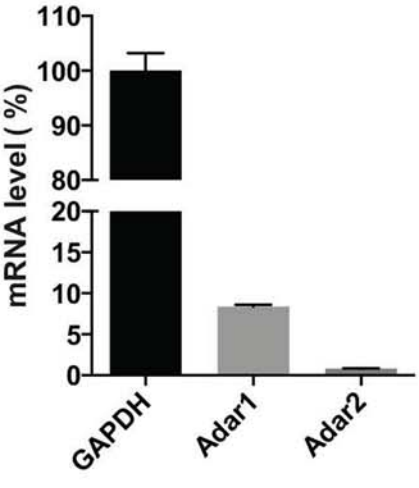


Figure.6D

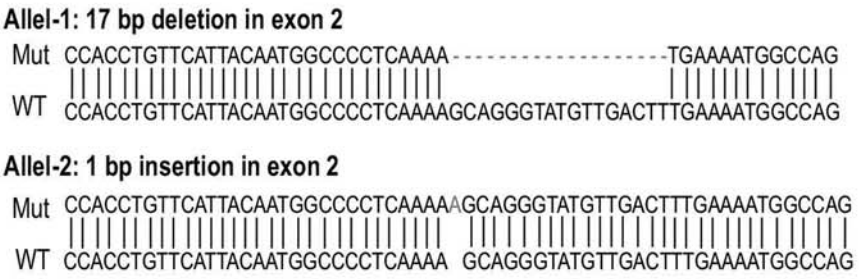


Figure.6E

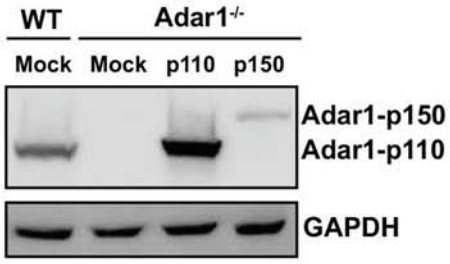


Figure.6F

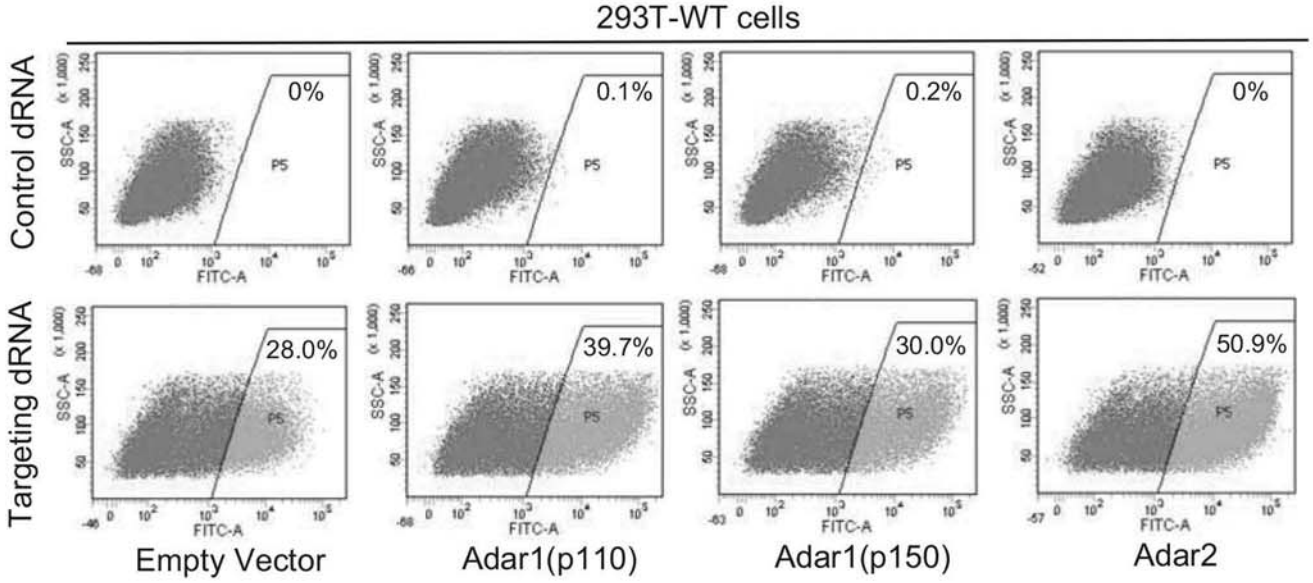


Figure.6G

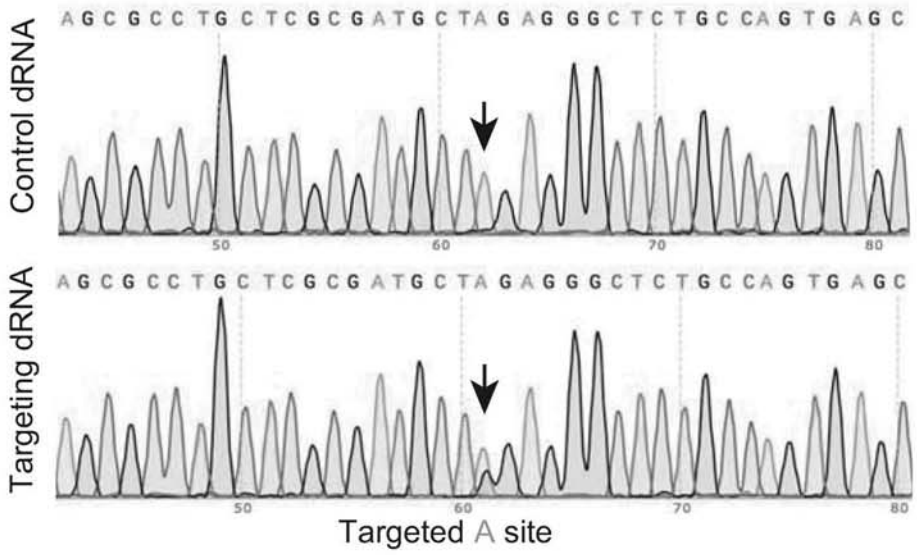


Figure.6H

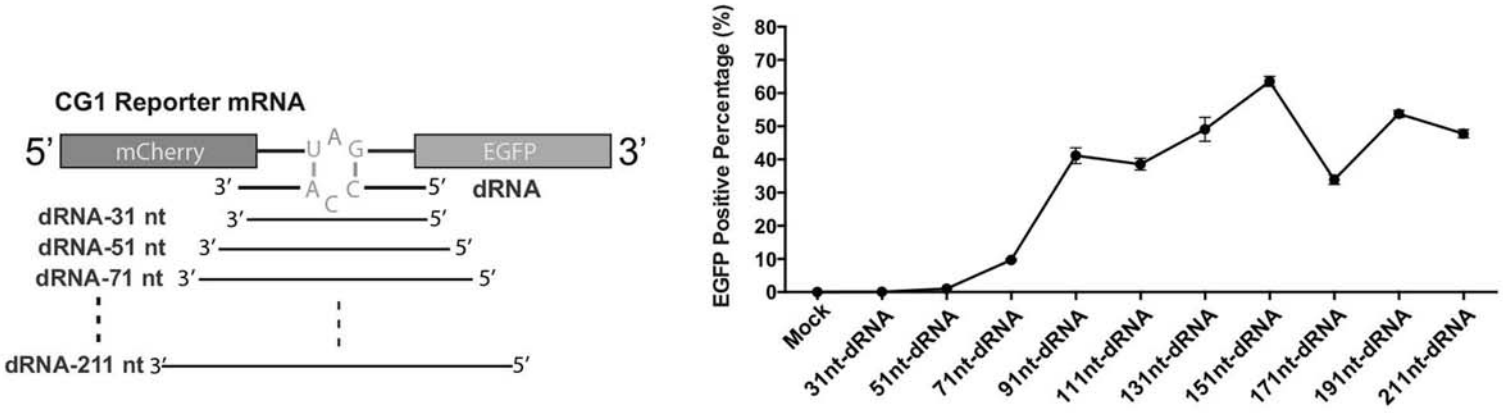


Figure.7A

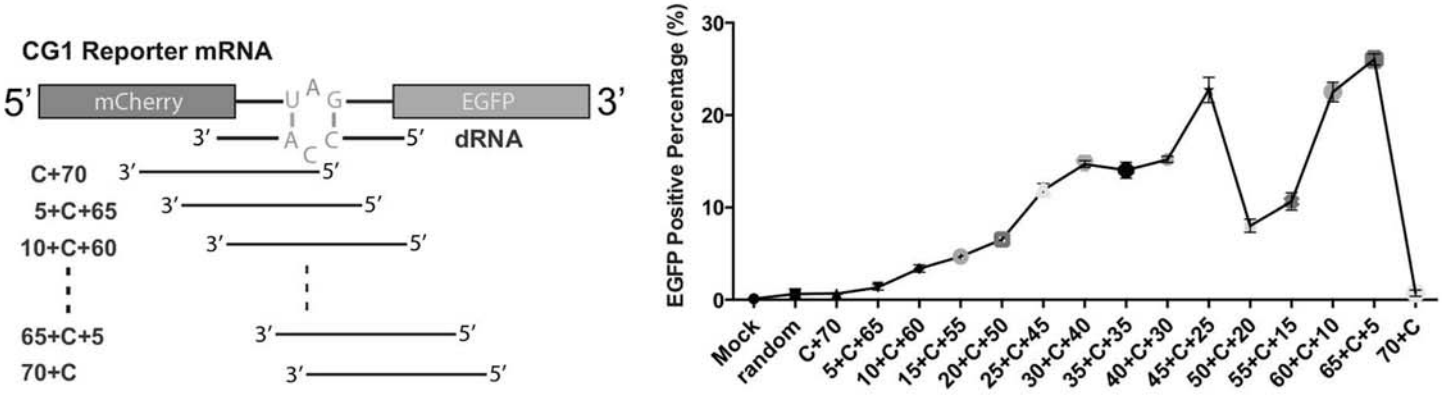


Figure.7B

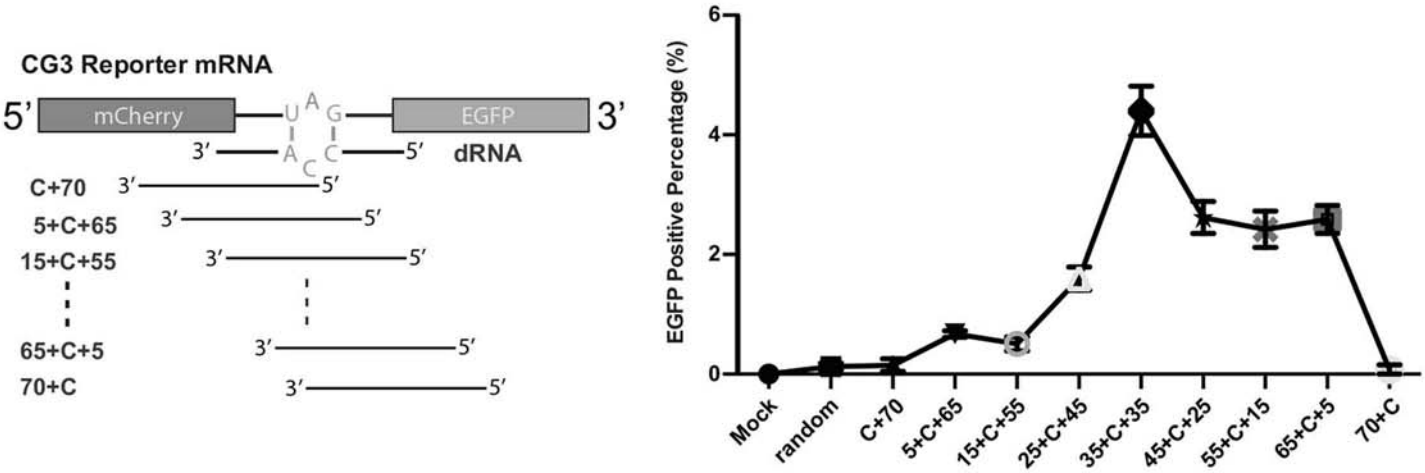


Figure.7C

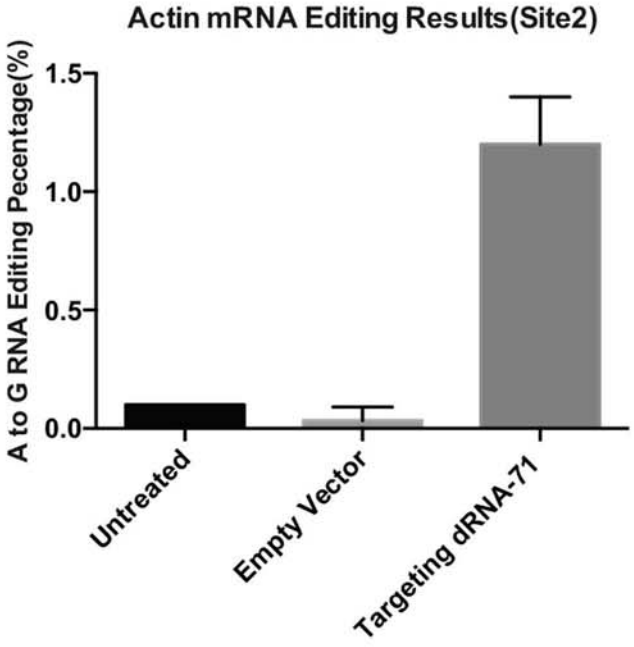


Figure.8A

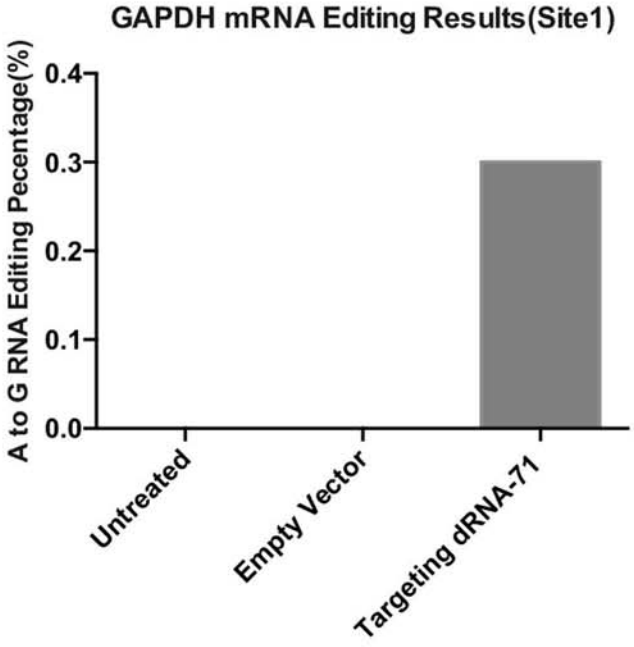


Figure.8B

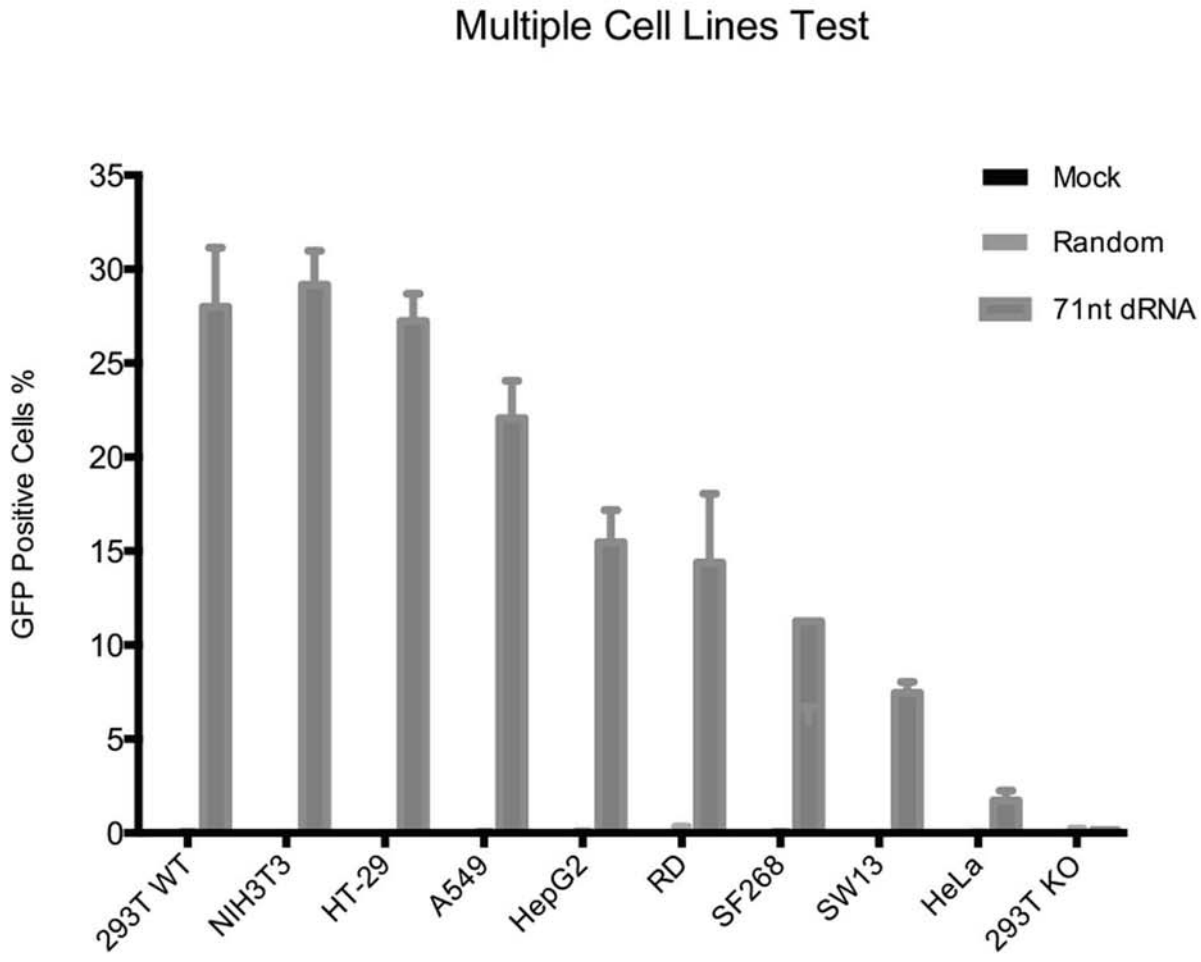

Multiple Cell Lines Test
GFP Positive Cells %
35
30
25
20
15
10
5
0
Mock
Random
71nt dRNA
293T WT
NIH3T3
HT-29
A549
HepG2
RD
SF268
SW13
HeLa
293T KO

Figure.9

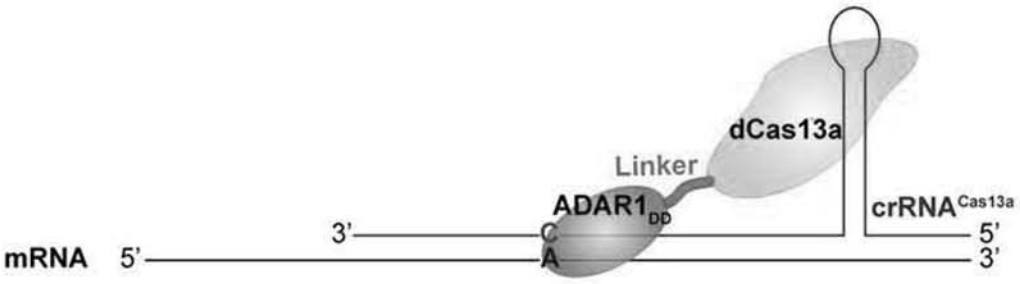


Figure.10A

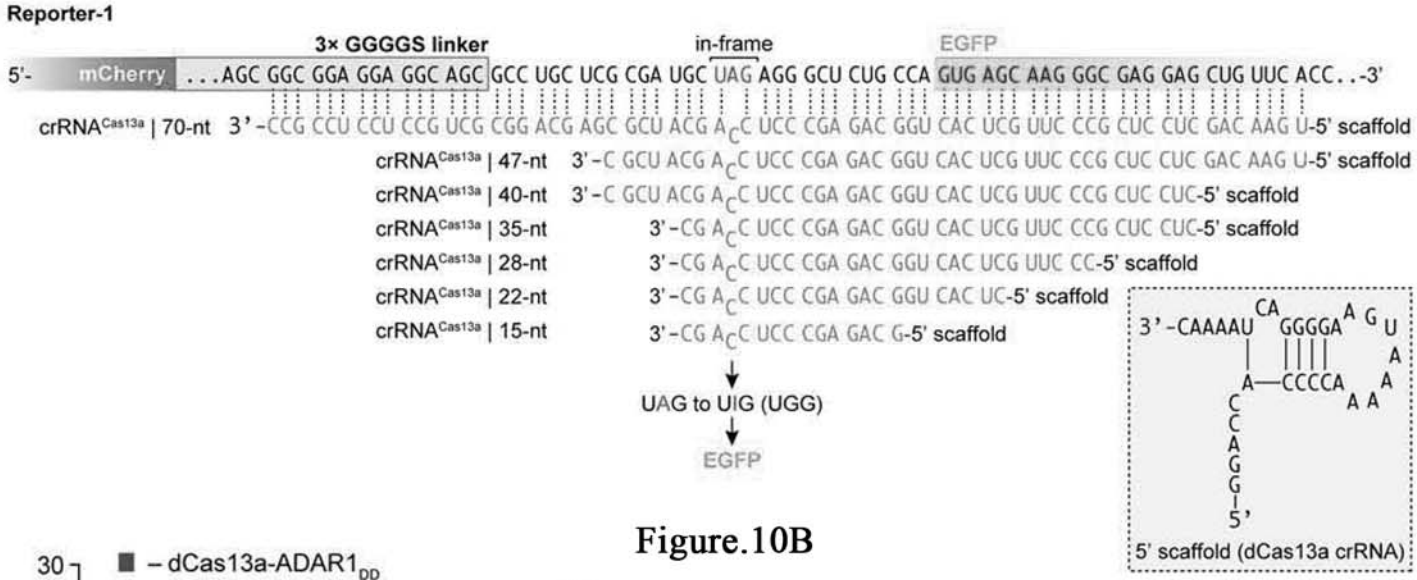


Figure.10B

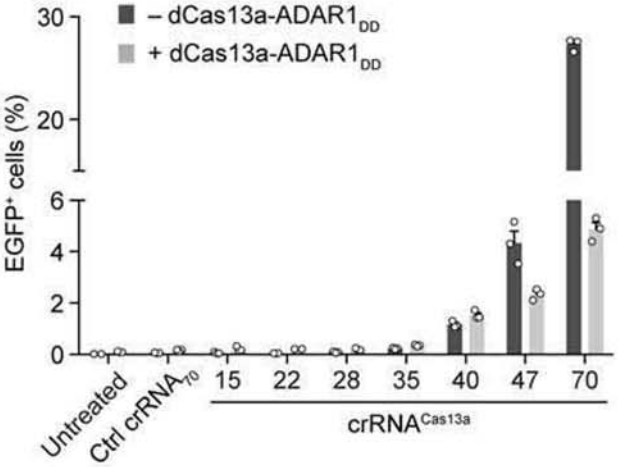


Figure.10C

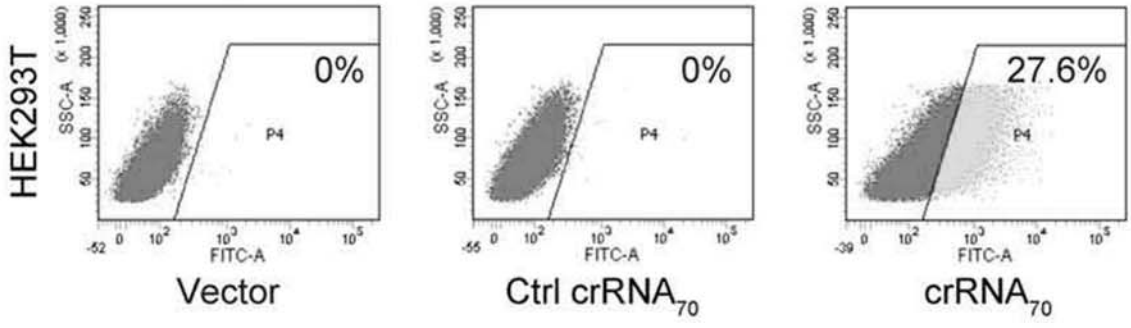


Figure.10D

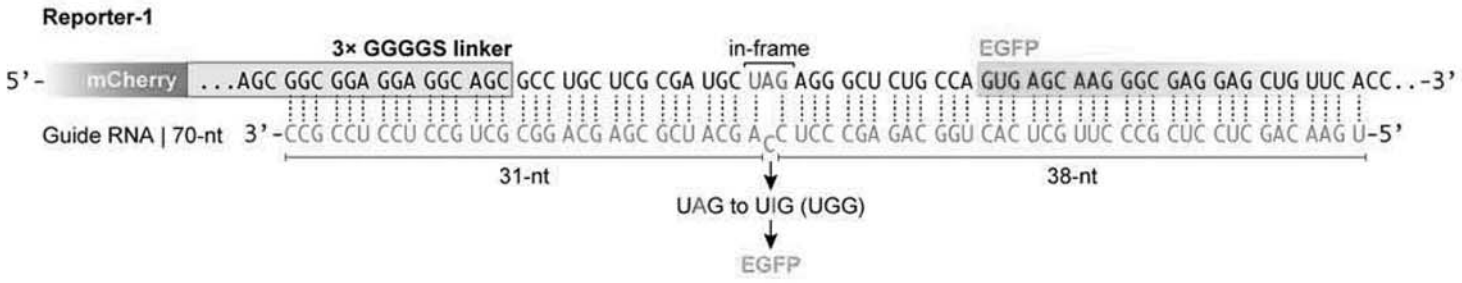


Figure.11A

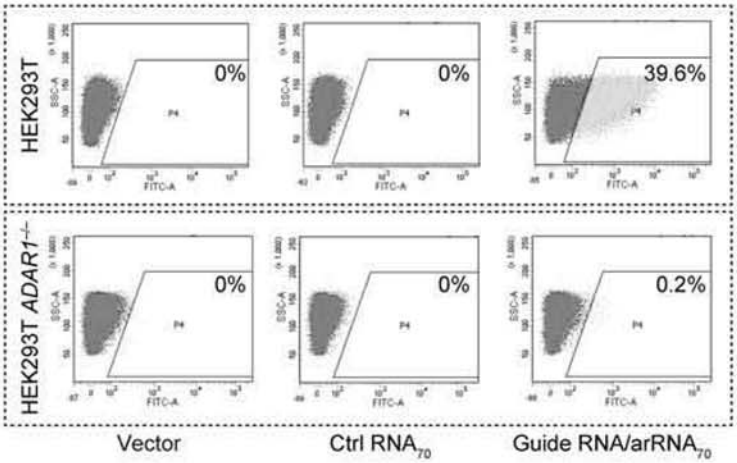


Figure.11B

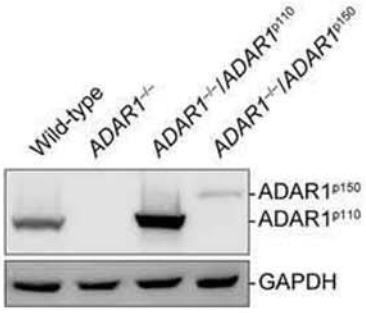


Figure.11C

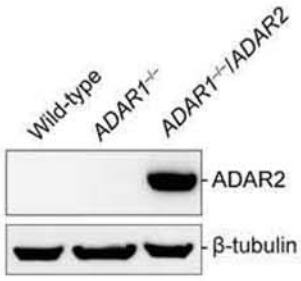


Figure.11D

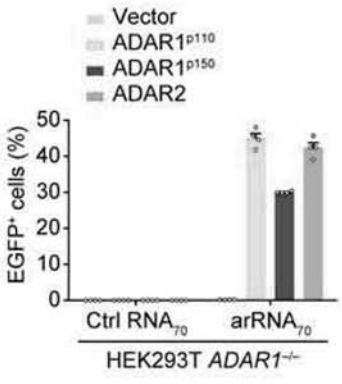


Figure.11E

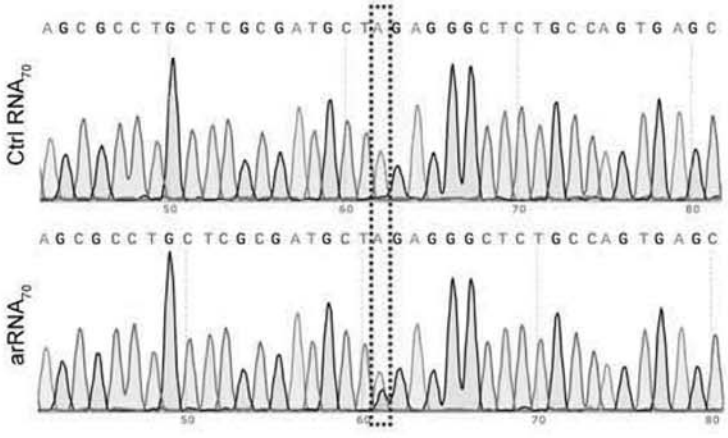


Figure.11F

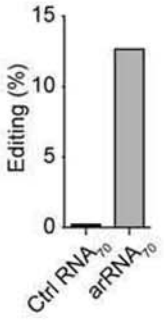


Figure.11G

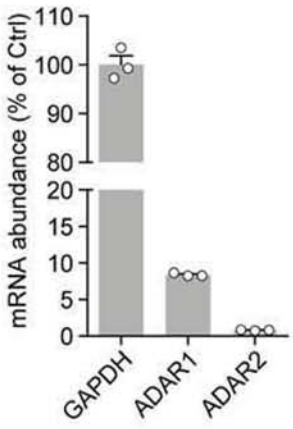


Figure.12A

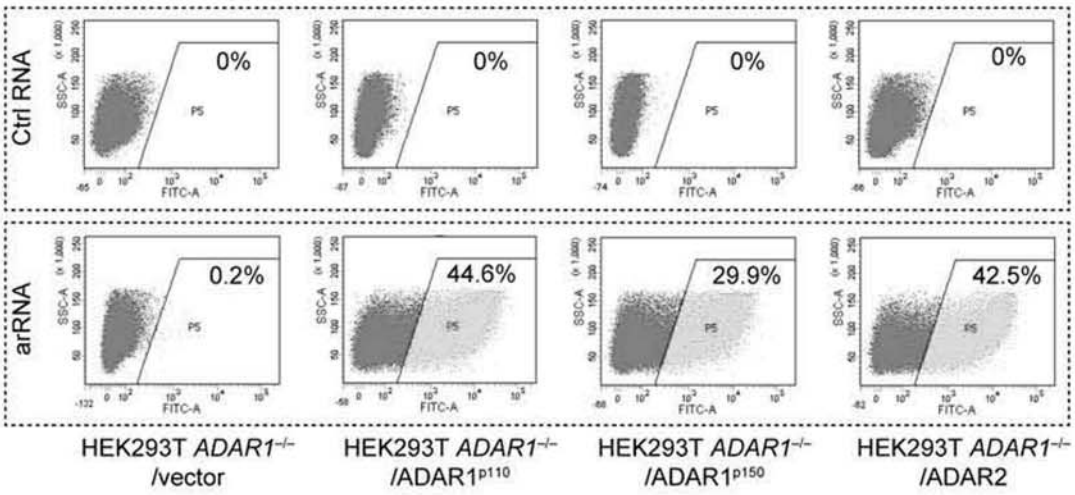


Figure.12B

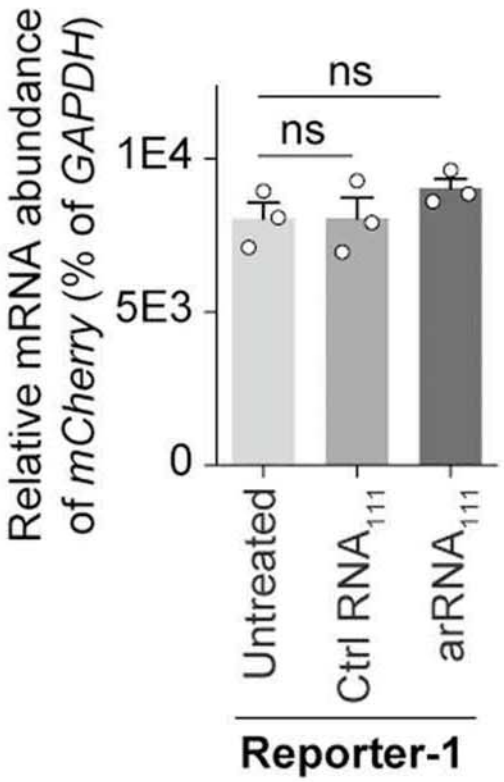


Figure.13

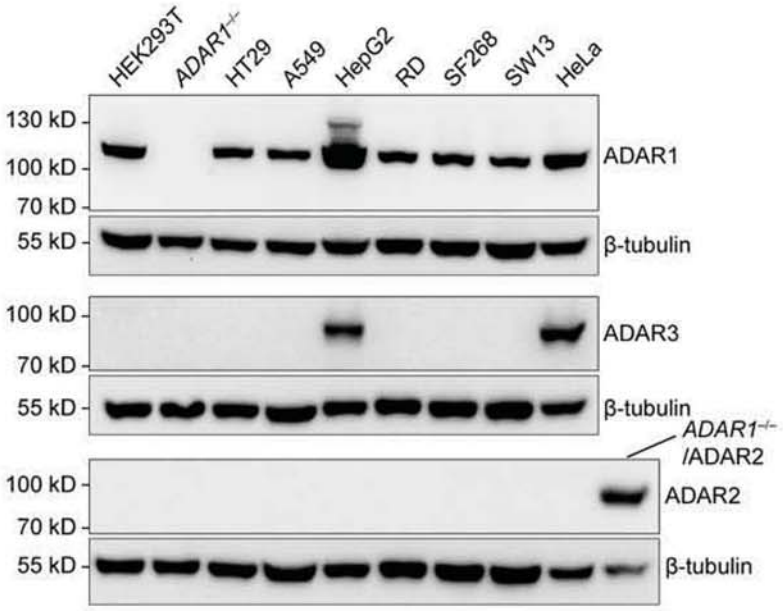


Figure.14A

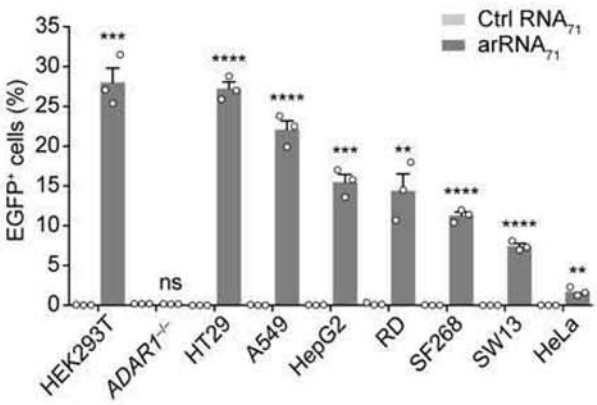


Figure.14C

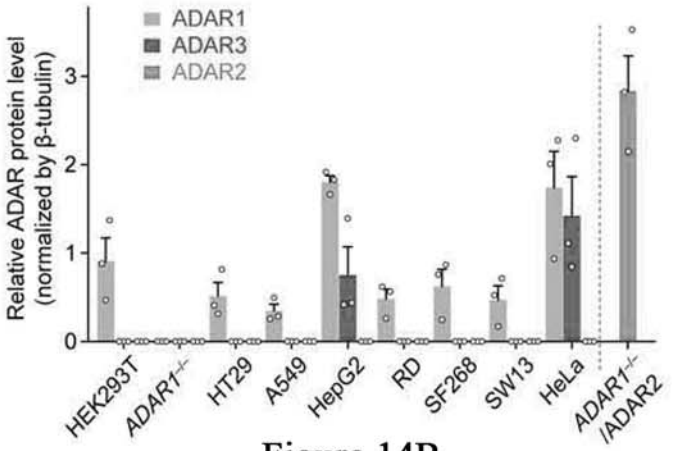


Figure.14B

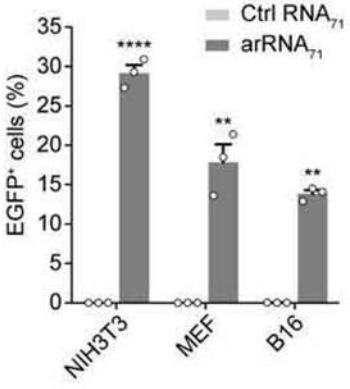


Figure.14D

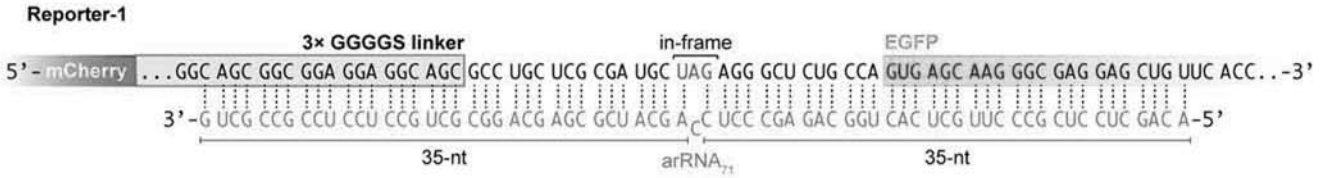


Figure.15A

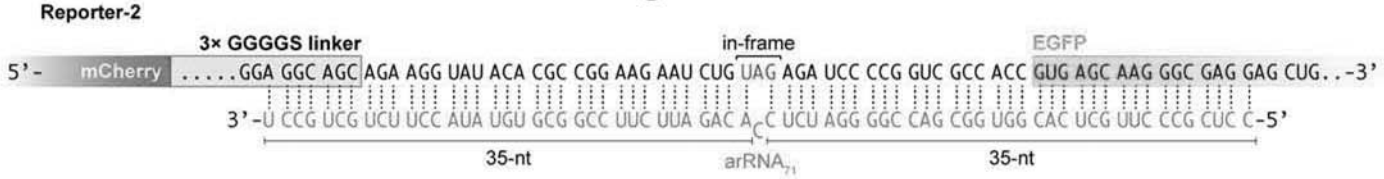


Figure.15B

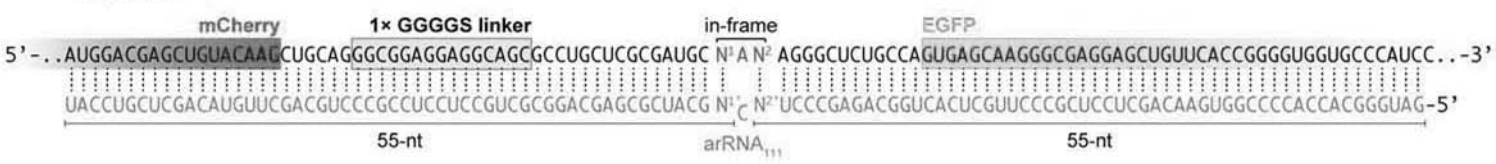


Figure.15C

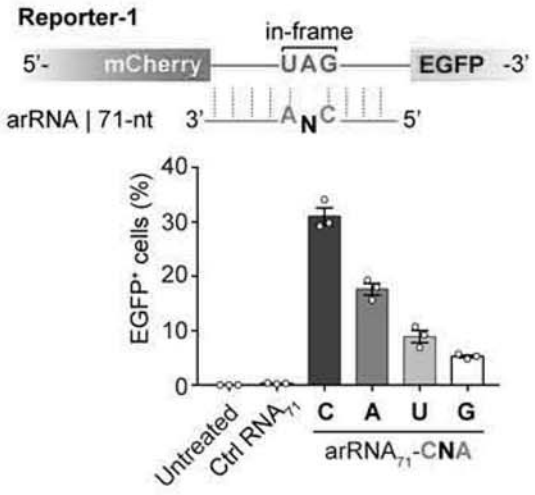


Figure.16A

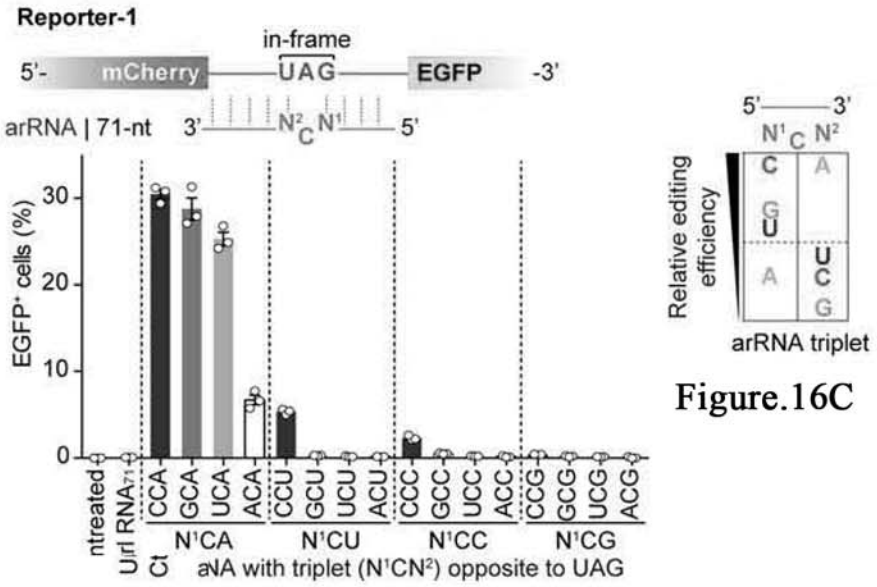


Figure.16C

Figure.16B

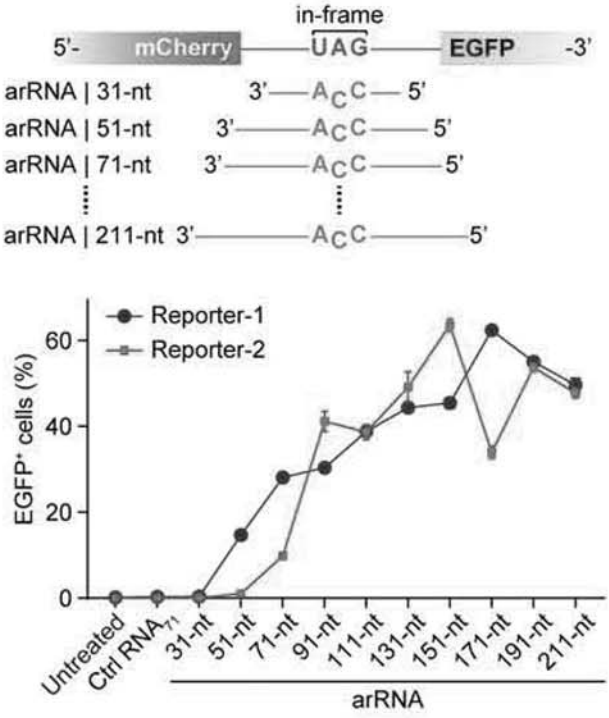


Figure.16D

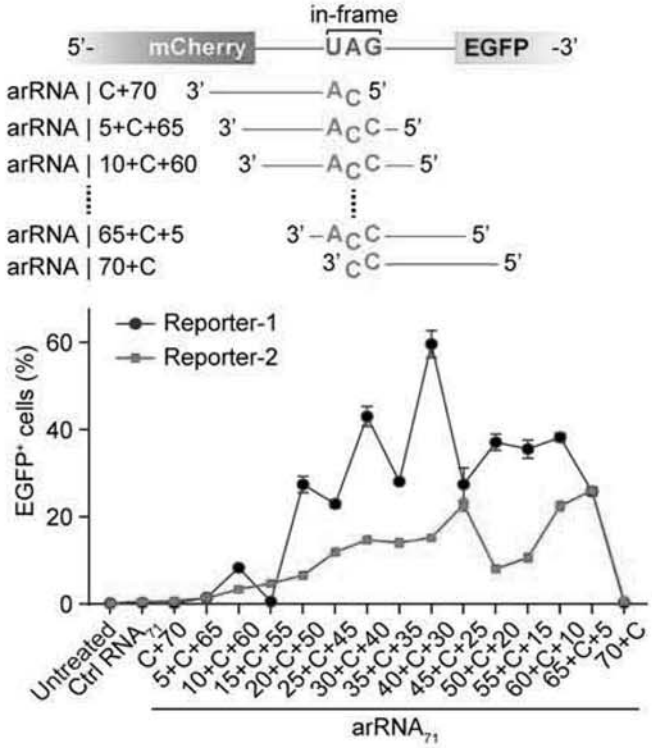


Figure.16E

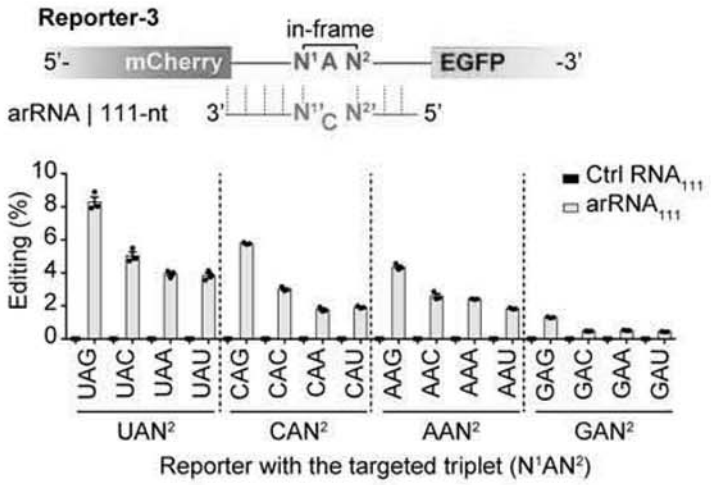


Figure.16F

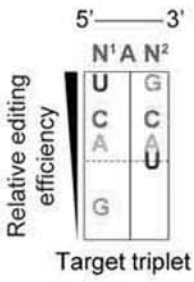


Figure.16G

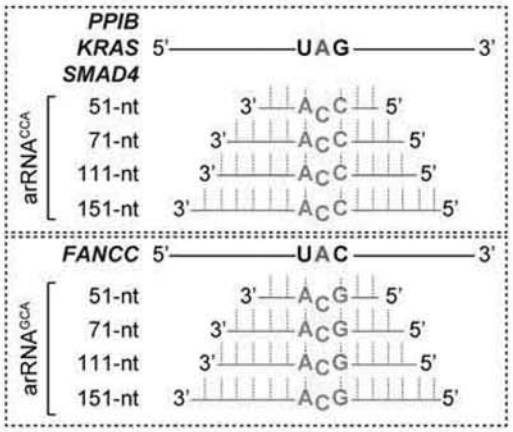


Figure.17A

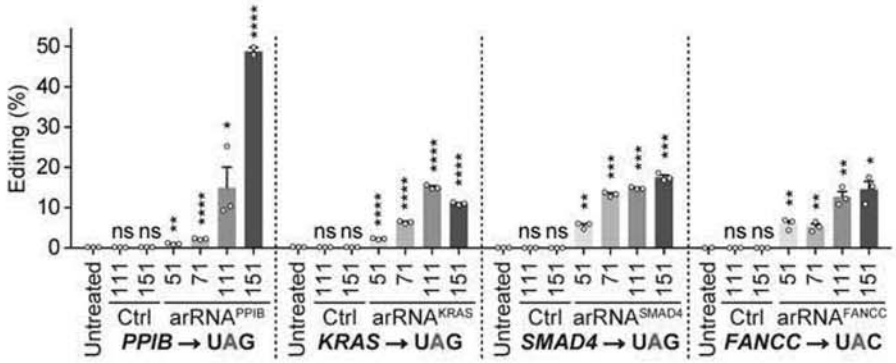


Figure.17B

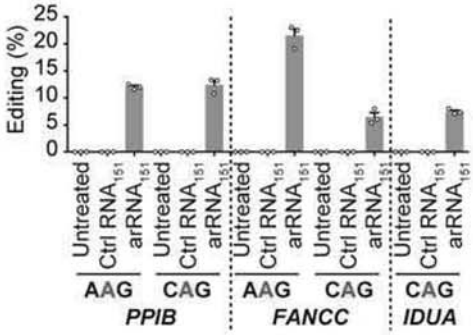


Figure.17C

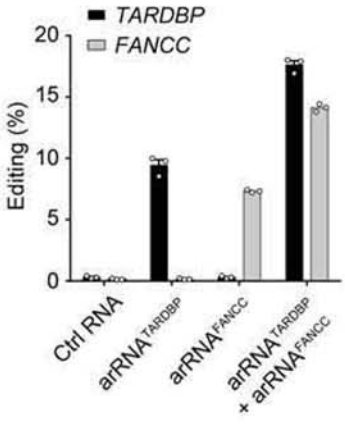


Figure.17D

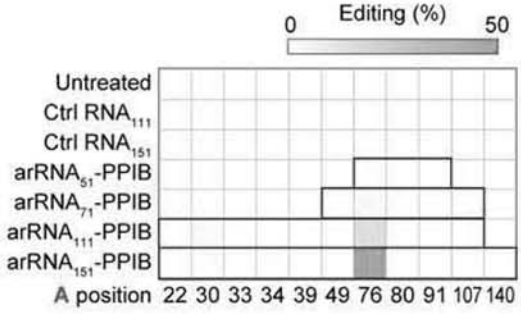


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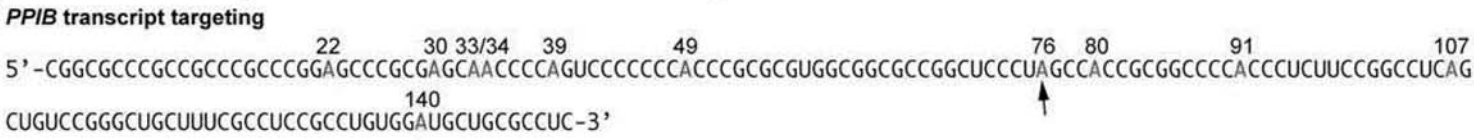


Figure.17E

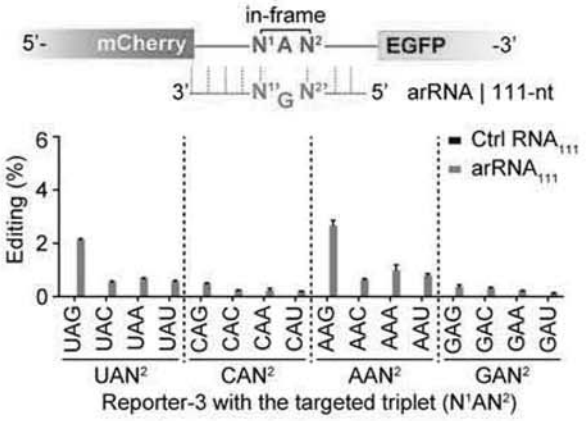


Figure.17G

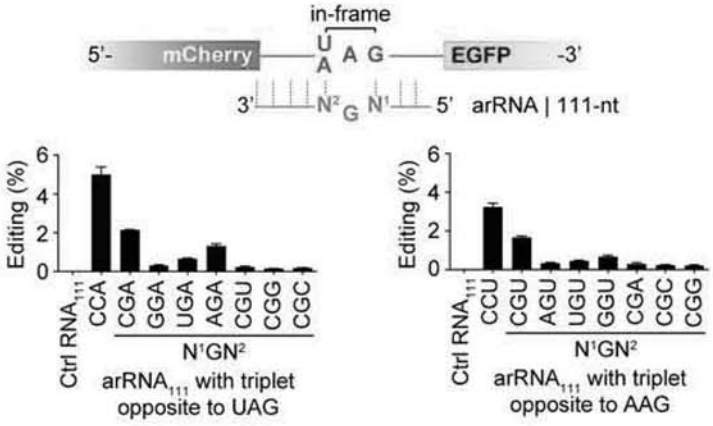


Figure.17H

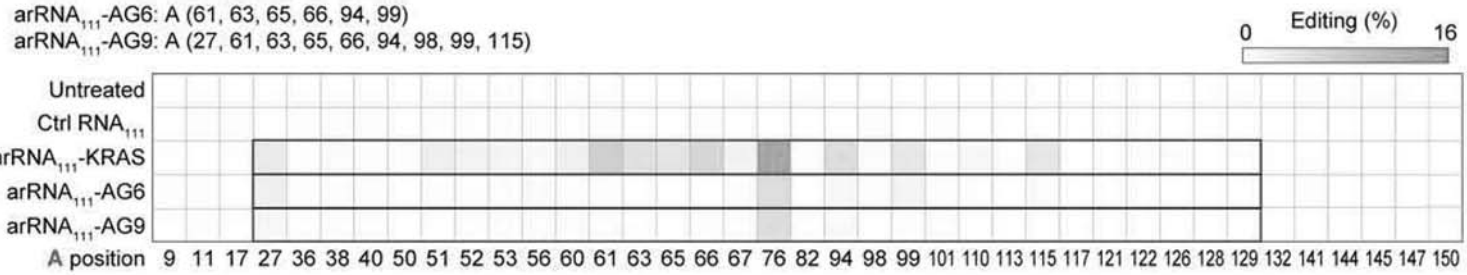


Figure.17I

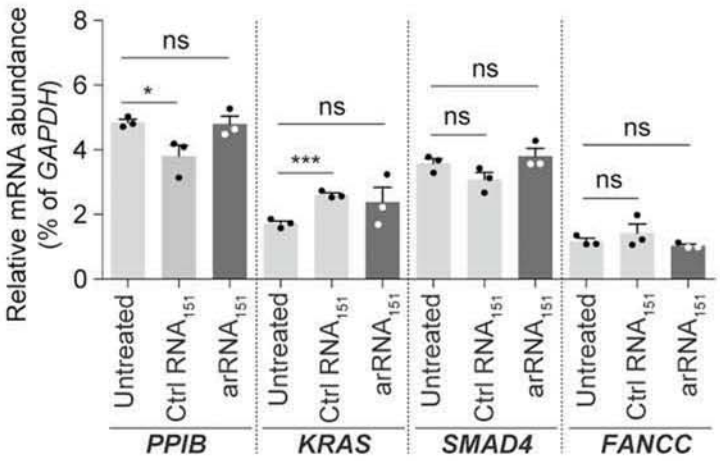


Figure.18A

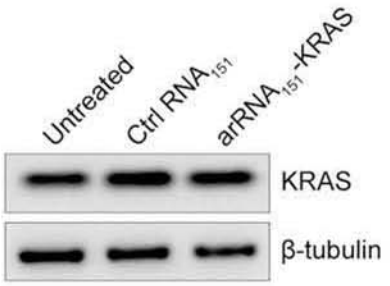


Figure.18B

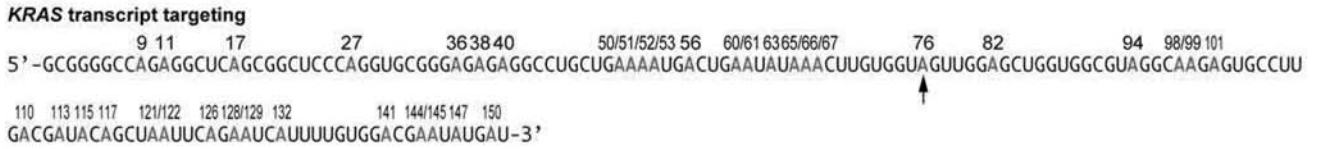


Figure.19A

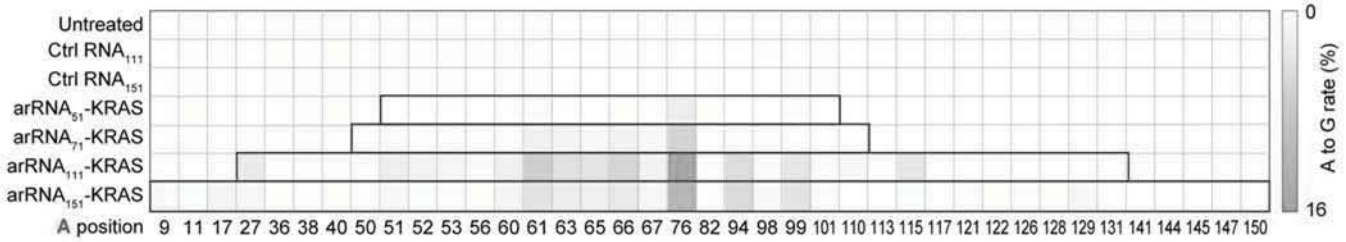


Figure.19B

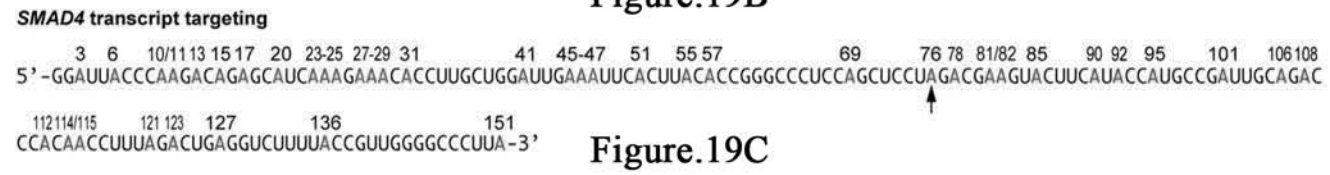


Figure.19C

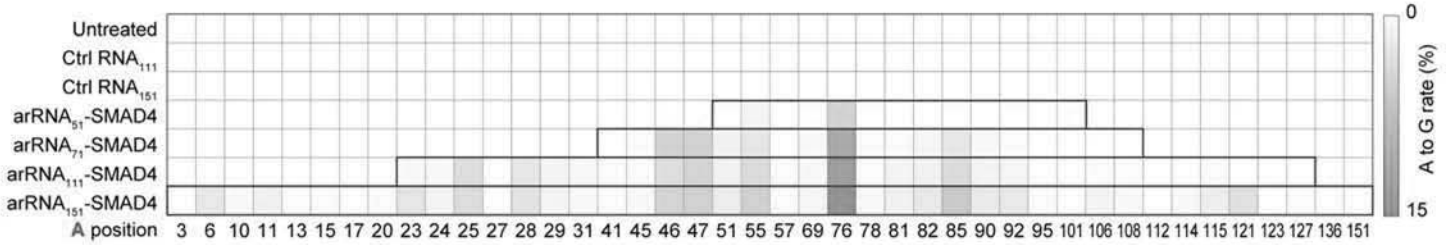


Figure.19D

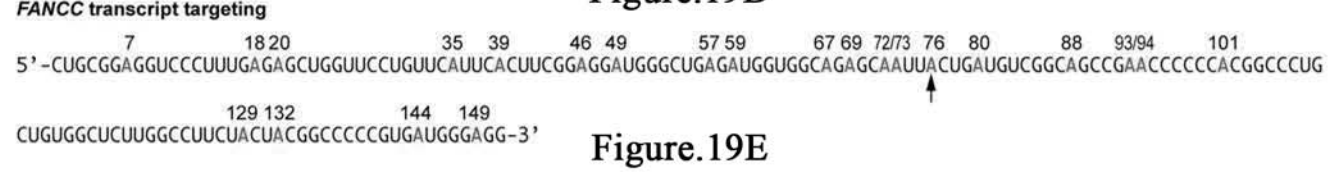


Figure.19E

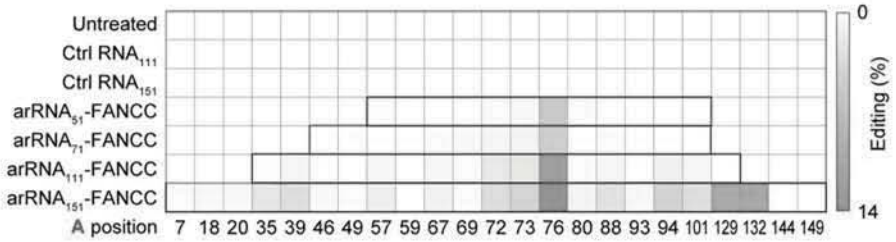


Figure.19F

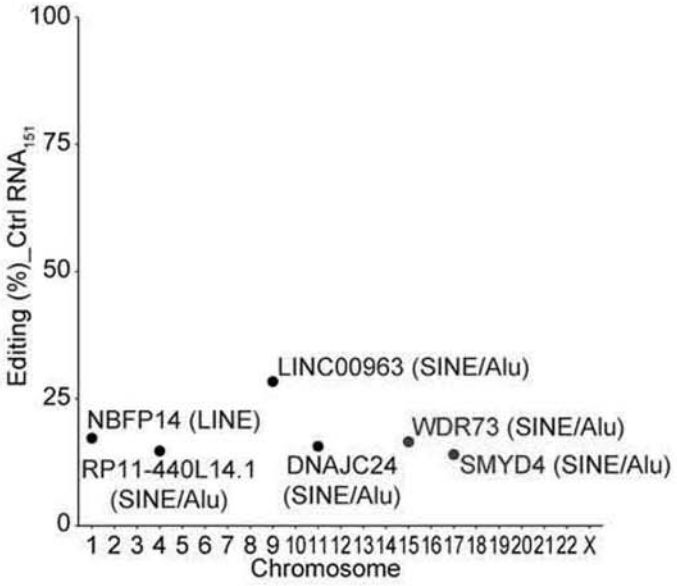


Figure.20A

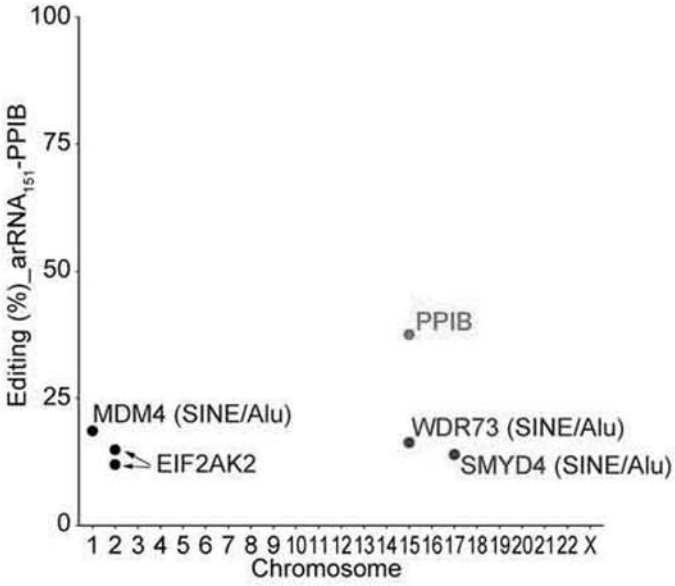


Figure.20B

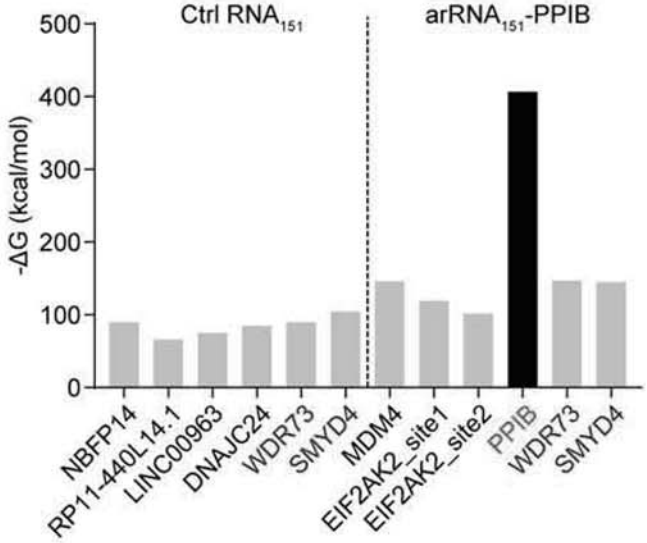


Figure.20C

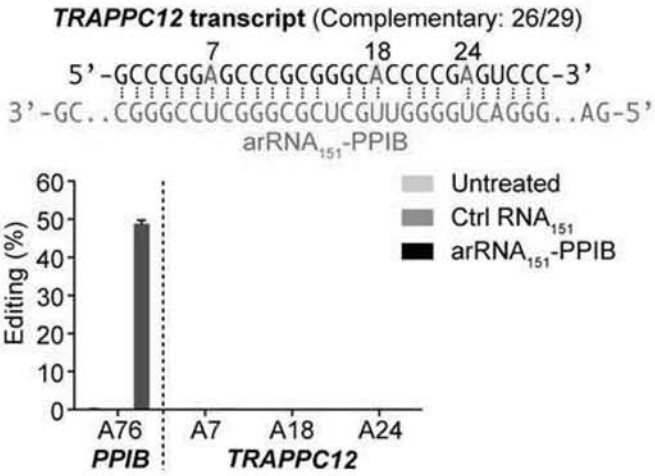


Figure.20D

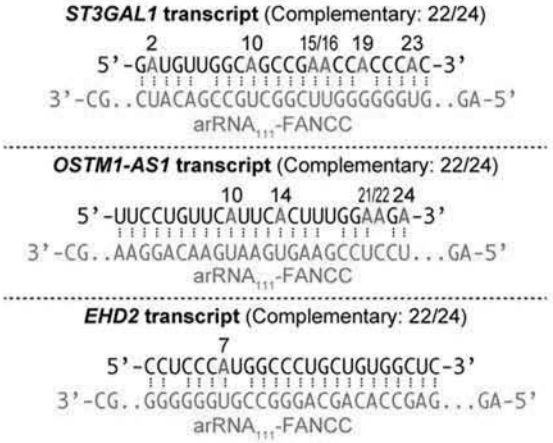


Figure.21A

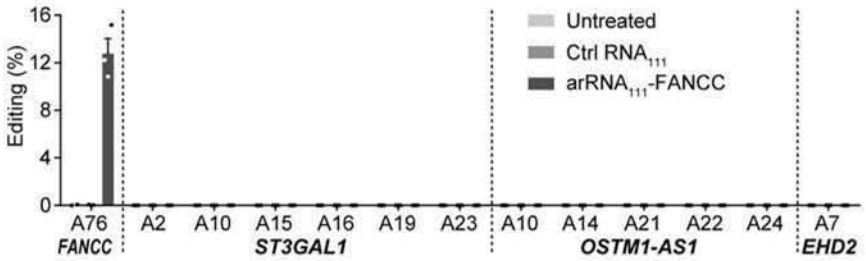


Figure.21B

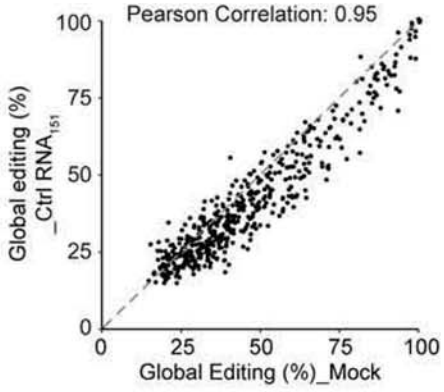


Figure.22A

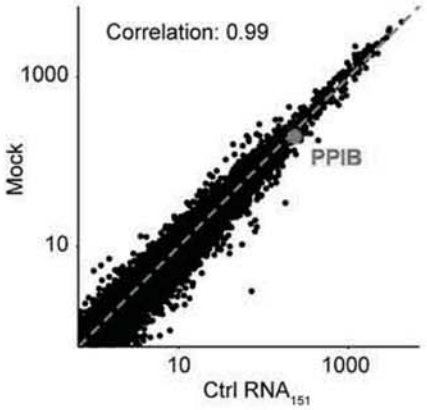


Figure.22C

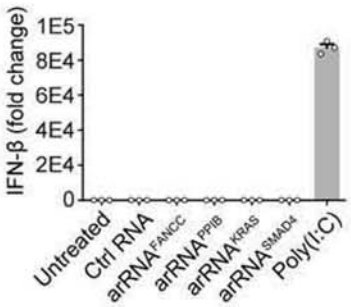


Figure.22E

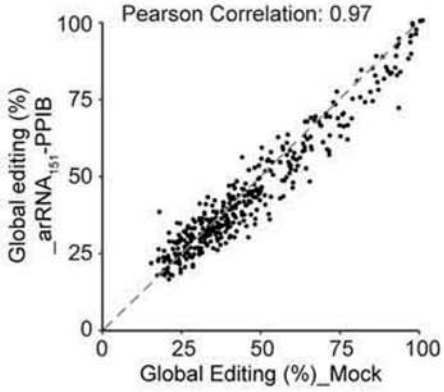


Figure.22B

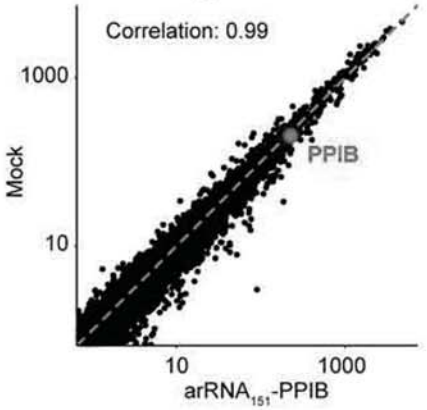


Figure.22D

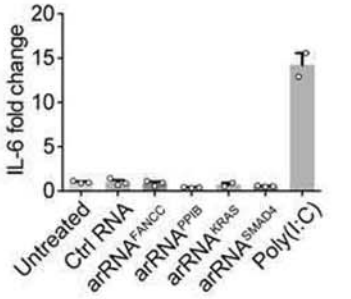


Figure.22F

***TP53^{W53X}* transcript targeting**

11/12 15/16 20 23 28
5'-UUGCCGUCCCAAGCAAUGGAUGAUUUGAUG

41 44 46 50/5153/54 56 61
CUGUCCCCGGACGAUAUUGAACAAUAGUUCACU

65/6668 72 78 80 83/84 91 93/94
GAAGACCCAGGUCCAGAUGAAGCUCCCAGAAUG

99 101
CCAGAGGCUGCUCCC-3'

arRNA$_{111}$-AG1: A (46)
arRNA$_{111}$-AG4: A (16, 46, 91, 94)

Figure.23A

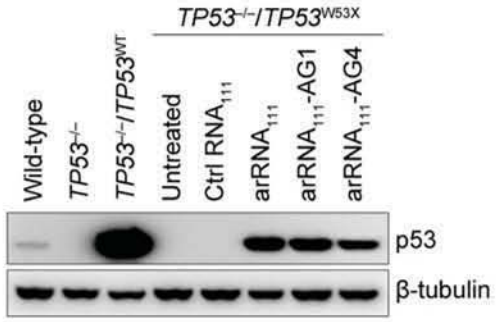


Figure.23C

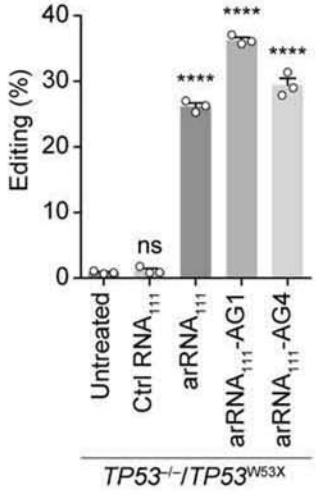


Figure.23B

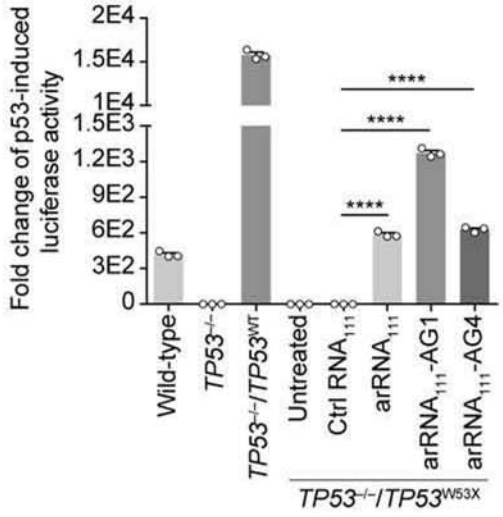


Figure.23D

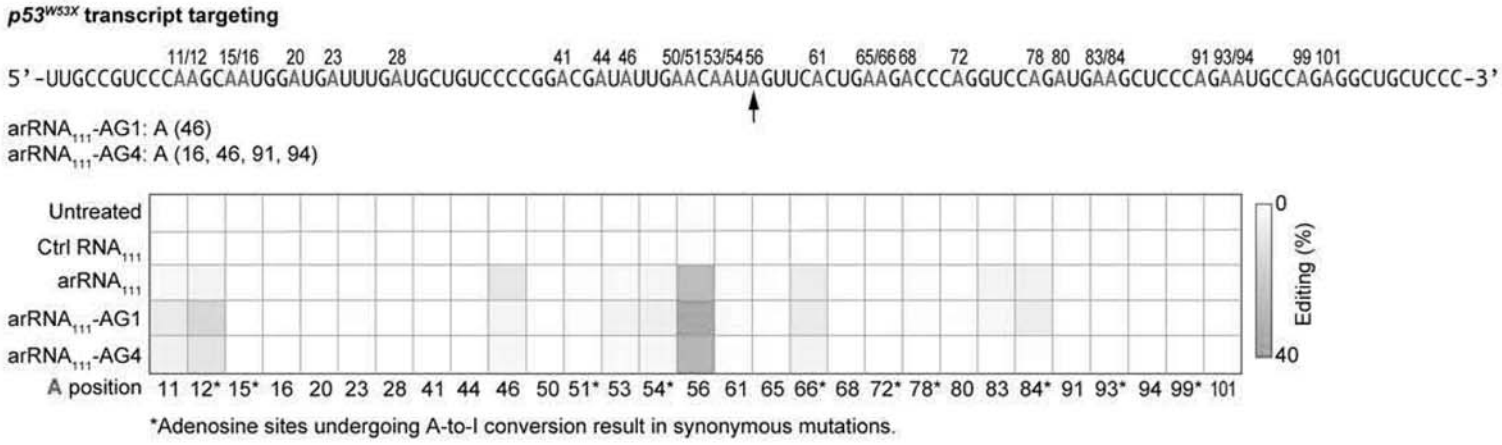


Figure.24

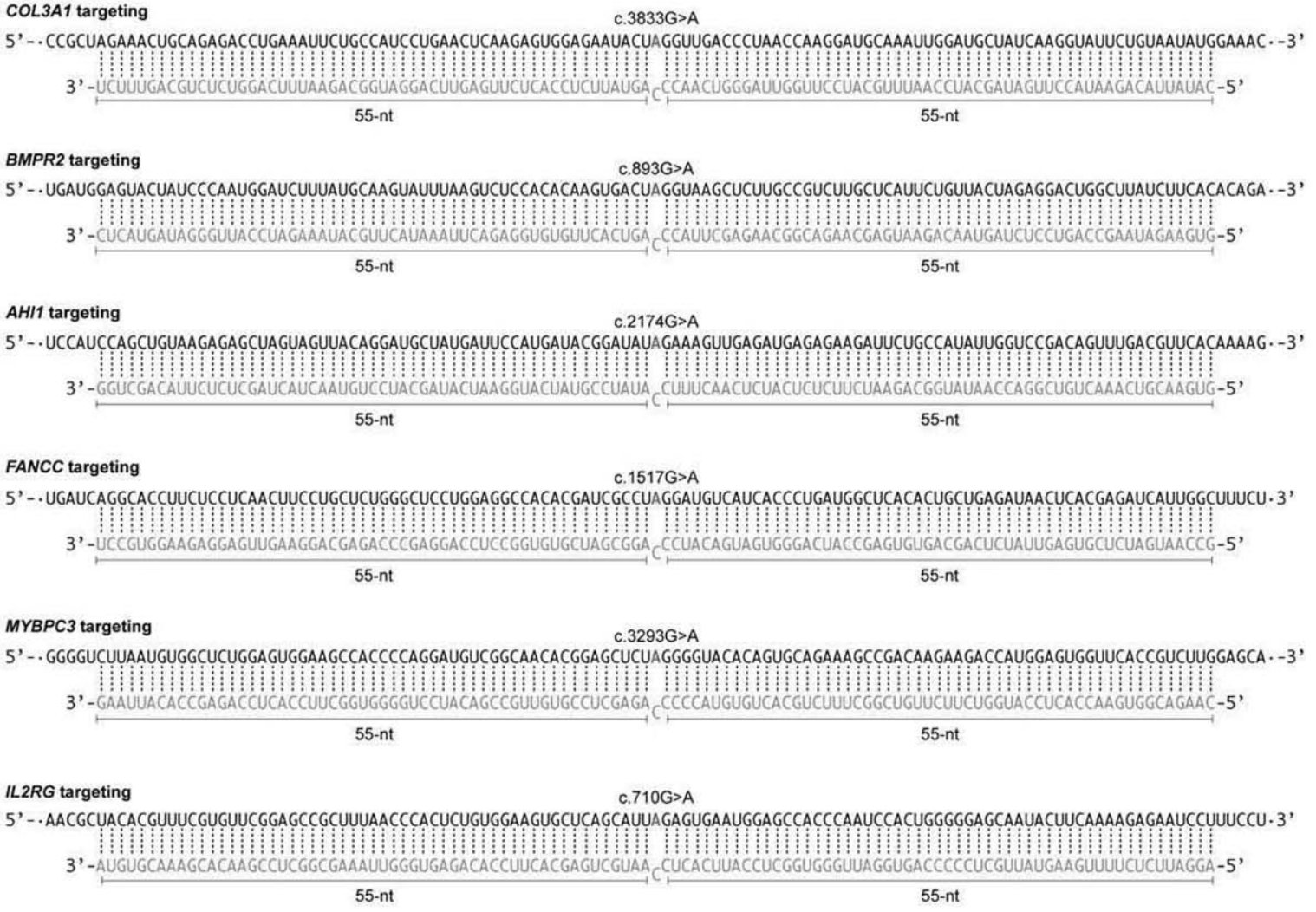


Figure.25

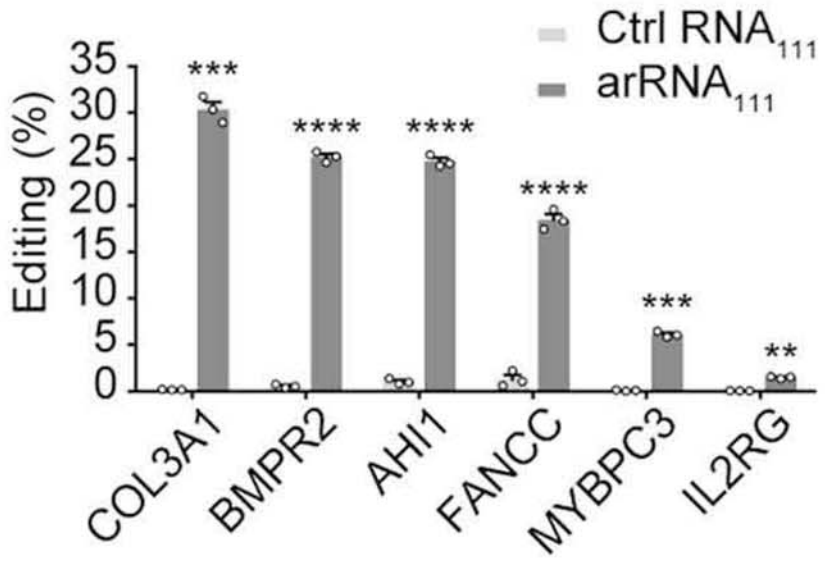


Figure.26

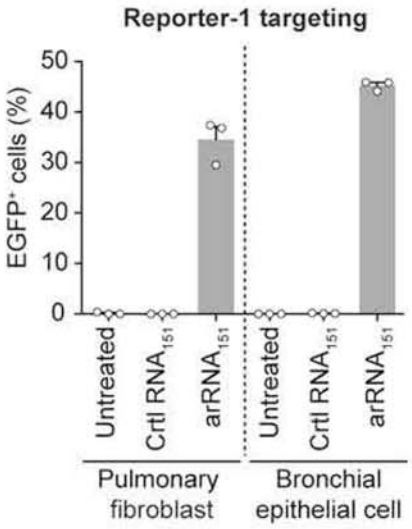


Figure.27A

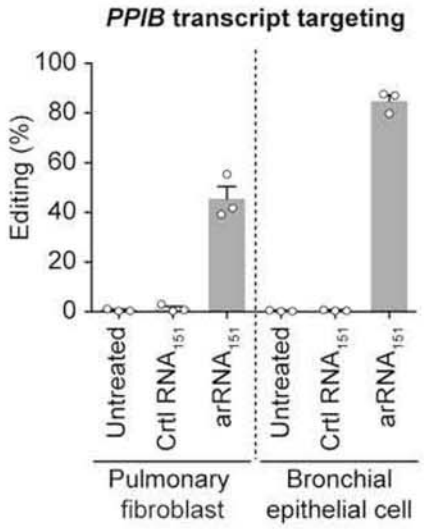


Figure.27B

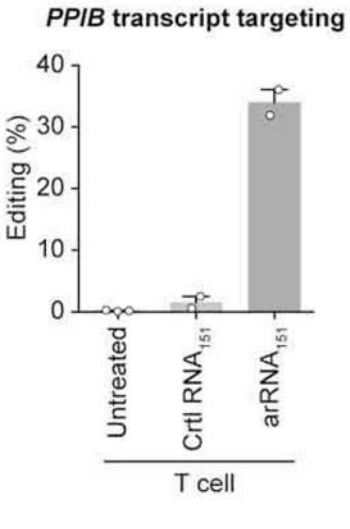


Figure.27C

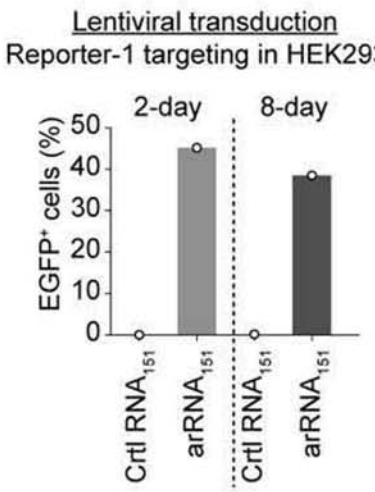


Figure.28A

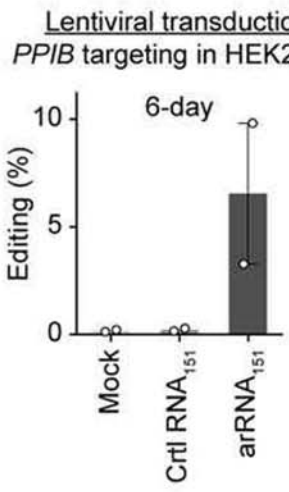


Figure.28B

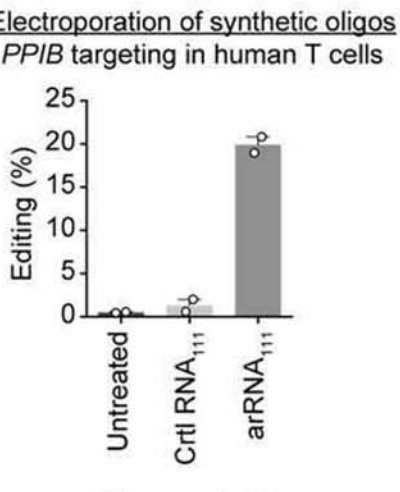


Figure.28D

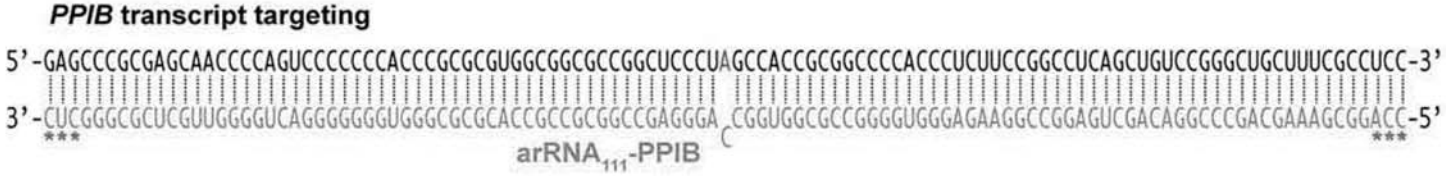


Figure.28C

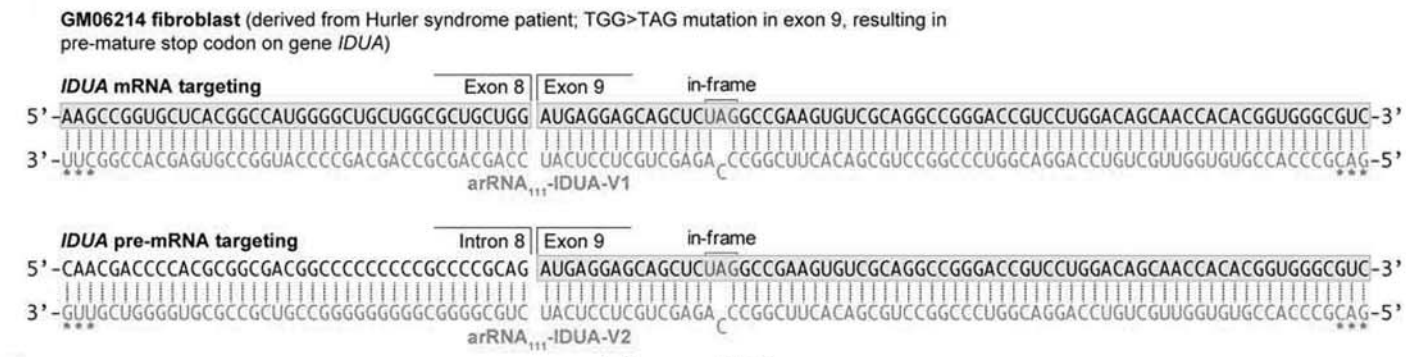


Figure.29A

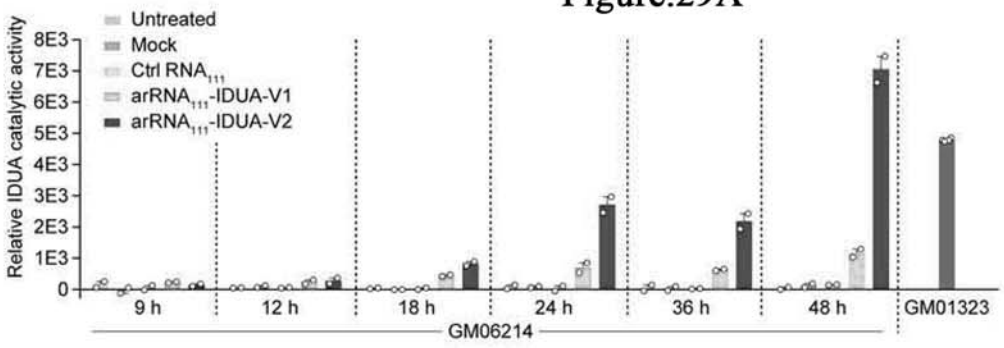


Figure.29B

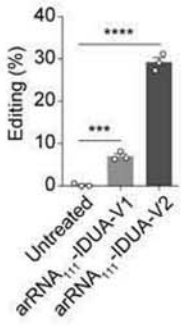


Figure.29C

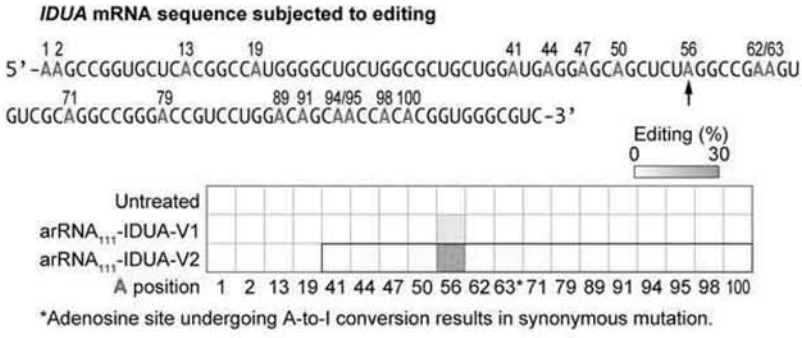


Figure.29D

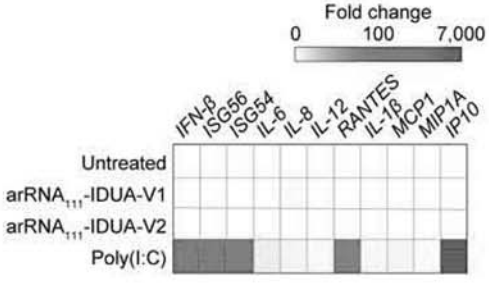


Figure.29E

Reporter-1: (SEQ ID NO:1,SEQ ID NO:2, and SEQ ID NO:3)



5'-
ATGGTGAGCAAGGGCGAGGAGGATAACATGGCCATCATCAAGGAGTTCATGCGCTTCAAGGTGCA
CATGGAGGGCTCCGTGAACGGCCACGAGTTCGAGATCGAGGGCGAGGGCGAGGGCCGCCCCTAC
GAGGGCACCCAGACCGCCAAGCTGAAGGTGACCAAGGGTGGCCCCCTGCCCTTCGCCTGGGACAT
CCTGTCCCCTCAGTTCATGTACGGCTCCAAGGCCTACGTGAAGCACCCCGCCGACATCCCCGACTA
CTTGAAGCTGTCCTTCCCCGAGGGCTTCAAGTGGGAGCGCGTGATGAACTTCGAGGACGGCGGCG
TGGTGACCGTGACCCAGGACTCCTCCCTGCAGGACGGCGAGTTCATCTACAAGGTGAAGCTGCGC
GGCACCAACTTCCCCTCCGACGGCCCCGTAATGCAGAAGAAGACCATGGGCTGGGAGGCCTCCTC
CGAGCGGATGTACCCCGAGGACGGCGCCCTGAAGGGCGAGATCAAGCAGAGGCTGAAGCTGAAG
GACGGCGGCCACTACGACGCTGAGGTCAAGACCACCTACAAGGCCAAGAAGCCCGTGCAGCTGCC
CGGCGCCTACAACGTCAACATCAAGTTGGACATCACCTCCCACAACGAGGACTACACCATCGTGGA
ACAGTACGAACGCGCCGAGGGCCGCCACTCCACCGGCGGCATGGACGAGCTGTACAAG ***CTGCAG***
GGCGGAGGAGGCAGC GGCGGAGGAGGCAGC GGCGGAGGAGGCAGC
GCCTGCTCGCGATGCTAGAGGGCTCTGCCA
GTGAGCAAGGGCGAGGAGCTGTTCACCGGGGTGGTGCCCATCCTGGTCGAGCTGGACGGCGACGTAAAC
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GCTTCAGCCGCTACCCCGACCACATGAAGCAGCACGACTTCTTCAAGTCCGCCATGCCCGAAGGCTACGT
CCAGGAGCGCACCATCTTCTTCAAGGACGACGGCAACTACAAGACCCGCGCCGAGGTGAAGTTCGAGGG
CGACACCCTGGTGAACCGCATCGAGCTGAAGGGCATCGACTTCAAGGAGGACGGCAACATCCTGGGGCA
CAAGCTGGAGTACAACTACAACAGCCACAACGTCTATATCATGGCCGACAAGCAGAAGAACGGCATCAA
GGTGAACTTCAAGATCCGCCACAACATCGAGGACGGCAGCGTGCAGCTCGCCGACCACTACCAGCAGAA
CACCCCCATCGGCGACGGCCCCGTGCTGCTGCCCGACAACCACTACCTGAGCACCCAGTCCGCCCTGAGC
AAAGACCCCAACGAGAAGCGCGATCACATGGTCCTGCTGGAGTTCGTGACCGCCGCCGGGATCACTCTC
GGCATGGACGAGCTGTACAAGTAA-3'

Figure.30A

Reporter-2: (SEQ ID NO:1,SEQ ID NO:4, and SEQ ID NO:3)



5'-
ATGGTGAGCAAGGGCGAGGAGGATAACATGGCCATCATCAAGGAGTTCATGCGCTTCAAGGTGCA
CATGGAGGGCTCCGTGAACGGCCACGAGTTCGAGATCGAGGGCGAGGGCGAGGGCCGCCCCTAC
GAGGGCACCCAGACCGCCAAGCTGAAGGTGACCAAGGGTGGCCCCCTGCCCTTCGCCTGGGACAT
CCTGTCCCCTCAGTTCATGTACGGCTCCAAGGCCTACGTGAAGCACCCCGCCGACATCCCCGACTA
CTTGAAGCTGTCCTTCCCCGAGGGCTTCAAGTGGGAGCGCGTGATGAACTTCGAGGACGGCGGCG
TGGTGACCGTGACCCAGGACTCCTCCCTGCAGGACGGCGAGTTCATCTACAAGGTGAAGCTGCGC
GGCACCAACTTCCCCTCCGACGGCCCCGTAATGCAGAAGAAGACCATGGGCTGGGAGGCCTCCTC
CGAGCGGATGTACCCCGAGGACGGCGCCCTGAAGGGCGAGATCAAGCAGAGGCTGAAGCTGAAG
GACGGCGGCCACTACGACGCTGAGGTCAAGACCACCTACAAGGCCAAGAAGCCCGTGCAGCTGCC
CGGCGCCTACAACGTCAACATCAAGTTGGACATCACCTCCCACAACGAGGACTACACCATCGTGGA
ACAGTACGAACGCGCCGAGGGCCGCCACTCCACCGGCGGCATGGACGAGCTGTACAAG CTGCAG
GGCGGAGGAGGCAGC GGCGGAGGAGGCAGC GGCGGAGGAGGCAGC
AGAAGGTATACACGCCGGAAGAATCTGTAGAGATCCCCGGTCGCCACC
GTGAGCAAGGGCGAGGAGCTGTTCACCGGGGTGGTGCCCATCCTGGTCGAGCTGGACGGCGACGTAAAC
GGCCACAAGTTCAGCGTGTCCGGCGAGGGCGAGGGCGATGCCACCTACGGCAAGCTGACCCTGAAGTTC
ATCTGCACCACCGGCAAGCTGCCCGTGCCCTGGCCCACCCTCGTGACCACCCTGACCTACGGCGTGCAGT
GCTTCAGCCGCTACCCCGACCACATGAAGCAGCACGACTTCTTCAAGTCCGCCATGCCCGAAGGCTACGT
CCAGGAGCGCACCATCTTCTTCAAGGACGACGGCAACTACAAGACCCGCGCCGAGGTGAAGTTCGAGGG
CGACACCCTGGTGAACCGCATCGAGCTGAAGGGCATCGACTTCAAGGAGGACGGCAACATCCTGGGGCA
CAAGCTGGAGTACAACTACAACAGCCACAACGTCTATATCATGGCCGACAAGCAGAAGAACGGCATCAA
GGTGAACTTCAAGATCCGCCACAACATCGAGGACGGCAGCGTGCAGCTCGCCGACCACTACCAGCAGAA
CACCCCCATCGGCGACGGCCCCGTGCTGCTGCCCGACAACCACTACCTGAGCACCCAGTCCGCCCTGAGC
AAAGACCCCAACGAGAAGCGCGATCACATGGTCCTGCTGGAGTTCGTGACCGCCGCCGGGATCACTCTC
GGCATGGACGAGCTGTACAAGTAA-3'

Figure.30B

Reporter-3: (SEQ ID NO:1,SEQ ID NO:5, and SEQ ID NO:3)



5'-
ATGGTGAGCAAGGGCGAGGAGGATAACATGGCCATCATCAAGGAGTTCATGCGCTTCAAGGTGCACATG
GAGGGCTCCGTGAACGGCCACGAGTTCGAGATCGAGGGCGAGGGCGAGGGCCGCCCCTACGAGGGCAC
CCAGACCGCCAAGCTGAAGGTGACCAAGGGTGGCCCCCTGCCCTTCGCCTGGGACATCCTGTCCCCTCAG
TTCATGTACGGCTCCAAGGCCTACGTGAAGCACCCCGCCGACATCCCCGACTACTTGAAGCTGTCCTTCC
CCGAGGGCTTCAAGTGGGAGCGCGTGATGAACTTCGAGGACGGCGGCGTGGTGACCGTGACCCAGGACT
CCTCCCTGCAGGACGGCGAGTTCATCTACAAGGTGAAGCTGCGCGGCACCAACTTCCCCTCCGACGGCCC
CGTAATGCAGAAGAAGACCATGGGCTGGGAGGCCTCCTCCGAGCGGATGTACCCCGAGGACGGCGCCCT
GAAGGGCGAGATCAAGCAGAGGCTGAAGCTGAAGGACGGCGGCCACTACGACGCTGAGGTCAAGACCA
CCTACAAGGCCAAGAAGCCCGTGCAGCTGCCCGGCGCCTACAACGTCAACATCAAGTTGGACATCACCT
CCCACAACGAGGACTACACCATCGTGGAACAGTACGAACGCGCCGAGGGCCGCCACTCCACCGGCGGCA
TGGACGAGCTGTACAAG CTGCAG ***GGCGGAGGAGGCAGC***
GCCTGCTCGCGATGCTAGAGGGCTCTGCCA
GTGAGCAAGGGCGAGGAGCTGTTCACCGGGGTGGTGCCCATCCTGGTCGAGCTGGACGGCGACGTAAAC
GGCCACAAGTTCAGCGTGTCCGGCGAGGGCGAGGGCGATGCCACCTACGGCAAGCTGACCCTGAAGTTC
ATCTGCACCACCGGCAAGCTGCCCGTGCCCTGGCCCACCCTCGTGACCACCCTGACCTACGGCGTGCAGT
GCTTCAGCCGCTACCCCGACCACATGAAGCAGCACGACTTCTTCAAGTCCGCCATGCCCGAAGGCTACGT
CCAGGAGCGCACCATCTTCTTCAAGGACGACGGCAACTACAAGACCCGCGCCGAGGTGAAGTTCGAGGG
CGACACCCTGGTGAACCGCATCGAGCTGAAGGGCATCGACTTCAAGGAGGACGGCAACATCCTGGGGCA
CAAGCTGGAGTACAACTACAACAGCCACAACGTCTATATCATGGCCGACAAGCAGAAGAACGGCATCAA
GGTGAACTTCAAGATCCGCCACAACATCGAGGACGGCAGCGTGCAGCTCGCCGACCACTACCAGCAGAA
CACCCCCATCGGCGACGGCCCCGTGCTGCTGCCCGACAACCACTACCTGAGCACCCAGTCCGCCCTGAGC
AAAGACCCCAACGAGAAGCGCGATCACATGGTCCTGCTGGAGTTCGTGACCGCCGCCGGGATCACTCTC
GGCATGGACGAGCTGTACAAGTAA-3'

Figure.30C

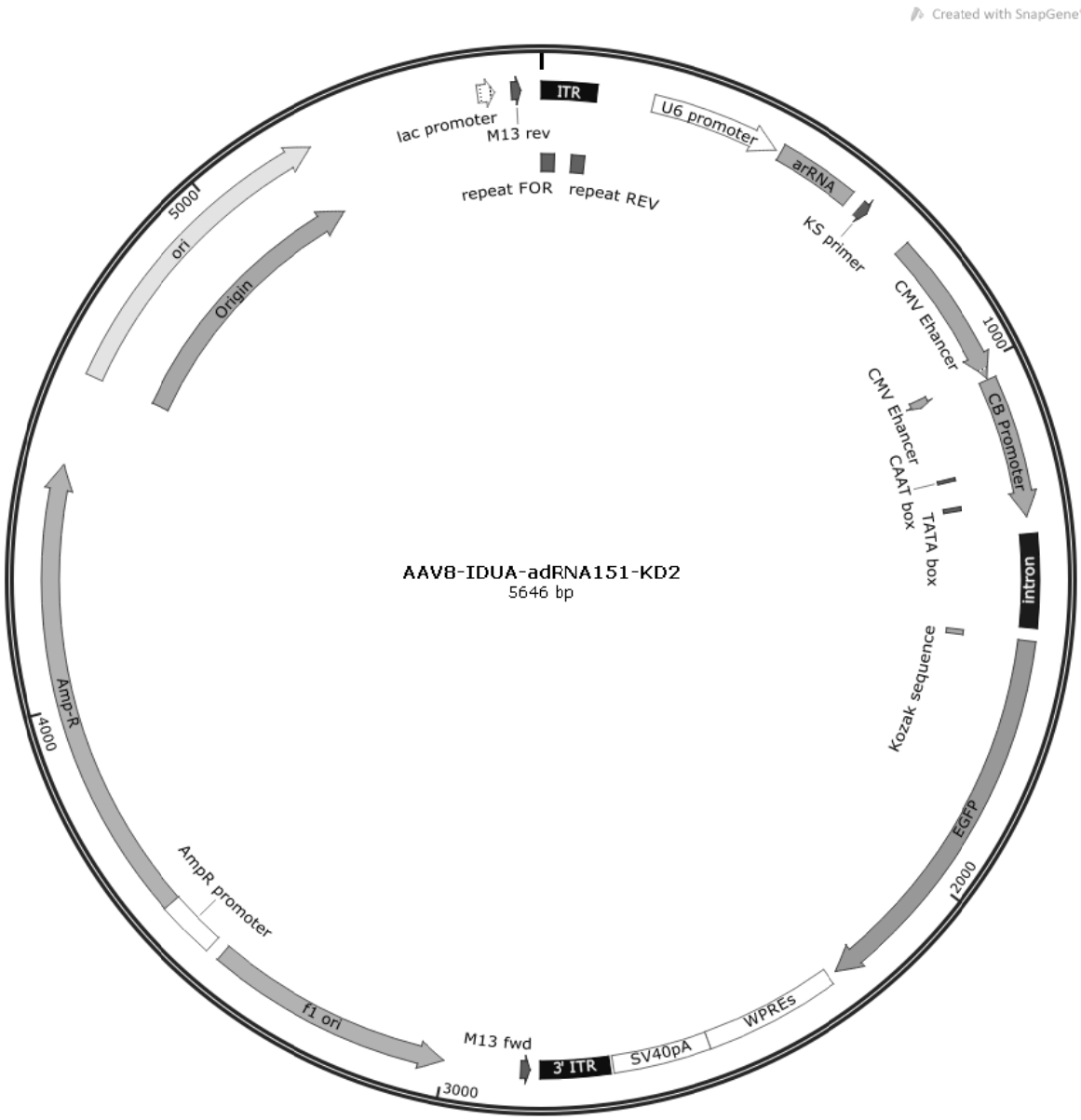

Created with SnapGene®
ITR
lac promoter
M13 rev
U6 promoter
arRNA
repeat FOR
repeat REV
KS primer
5000
ori
Origin
CMV Enhancer
1000
CB Promoter
CMV Enhancer
CAAT box
TATA box
intron
AAV8-IDUA-adRNA151-KD2
5646 bp
Kozak sequence
Amp-R
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EGFP
2000
AmpR promoter
f1 ori
M13 fwd
3' ITR
SV40pA
WPREs
3000

Figure.31

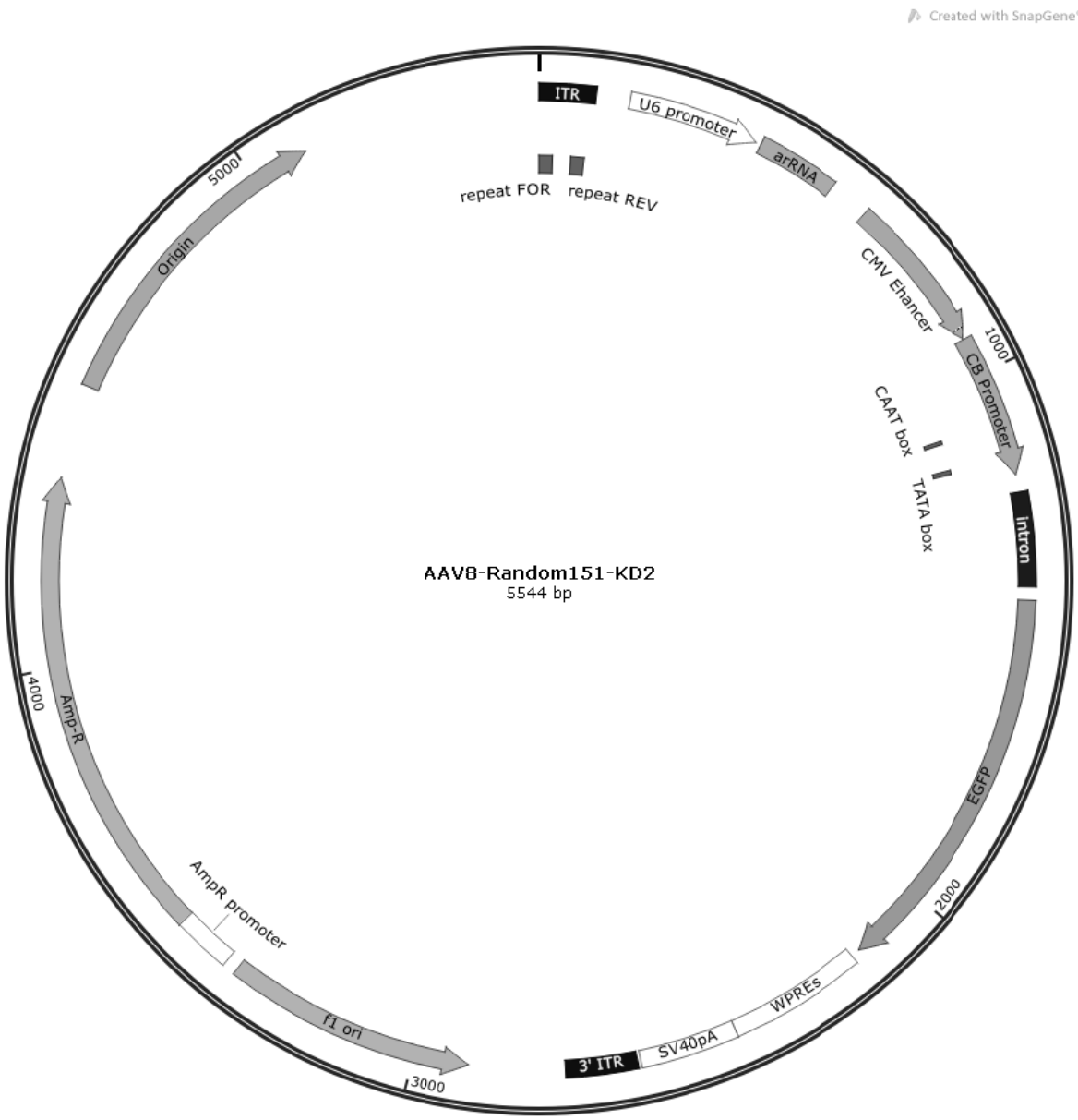


Figure.31(cont)

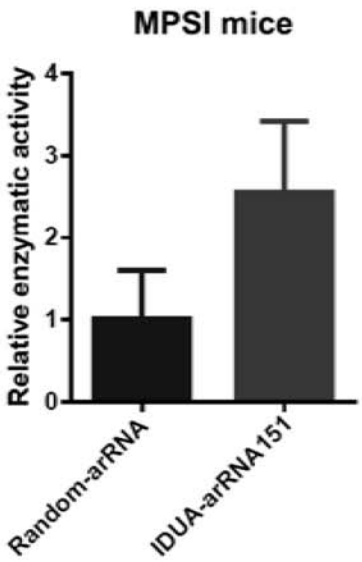


Figure.32A

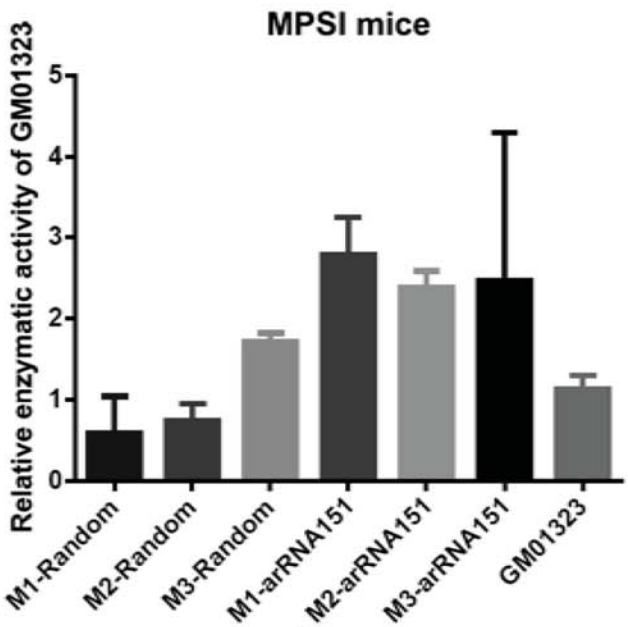


Figure.32B

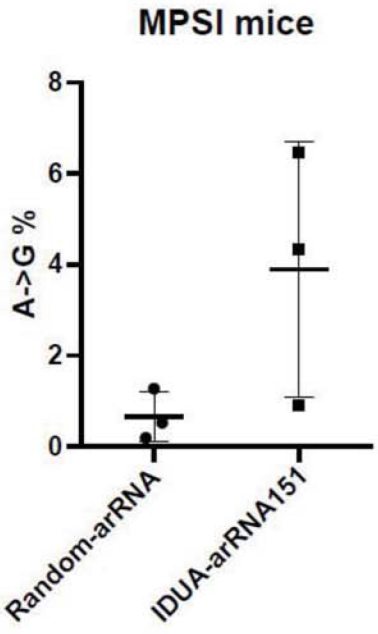


Figure.33A

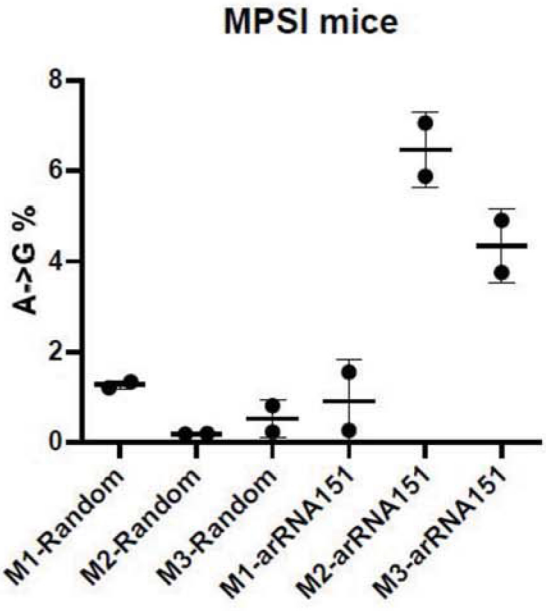


Figure.33B