

Codon optimized *NMNAT1* cDNA

1-75 ATGGAAATTCAGAGAAACTGAAGTGGT**GCTGTGGCATGTGGATCG**TTCAACCCCATCACCACATGCATCTG
 C G T CC T T T A T C C

76-150 CGCCTCTTTGAACTGGCCAAAGACTACATGAATGGAAGTGAAGATACACTGTGGTCAAAGGCATCATCTCCCA
 A T G G G A A T T T

151-225 GTGGGGATGCATACAAGAAGAAGGGCTTGATCCCTGCCTACCACCGGGTCATCATGGCTGAGCTGGCCACCAAG
 T T C A AC C T T A A T T

226-300 AACTCAAAATGGGTGGAAGTGGACACCTGGGAGTCACTGCAAAAGGAGTGAAGGAAACCTTAAAGTCTGCGG
 T T T T A AAGT T G A G T G G G A A A

301-375 CATCACCAGGAAAAGCTGGAAGCCTCGGACTGTGACCACCAGCAGAACAGCCCCACCCCTGAACGCCAGGGAGA
 C T A G AT G TAGT T TCA T T A A G T A G

376-450 AAGCGCAAGTGGACTGAGACCCAAGACTCAAGCCAGAAGAAGTCGCTGGAACCCAAGACCAAGCTGTCCCAAAG
 A G A A T T T A A C A G A A A G

451-525 GTGAAACTCCTCTGTGGAGCTGACCTCCTGGAATCGTTTGCTGTGCCTAATCTCTGGAAGTCGGAAGATATCACC
 C G G G G A TT AT G C T C T G AGT C

526-600 CAAATGTGGCCAACTATGGCCTGATCTGTGTGACTAGAGCTGGTAATGATGCCAGAAAATTCATCTATGAATCA
 C G C A T C G A T G T G

601-675 GATGTGCTGTGGAAGCACCGGAGCAACATCCATGTGGTCAATGAGTGGATTGCAAAATGACATCTCTCCACCAAG
 A T C G A C T A A A

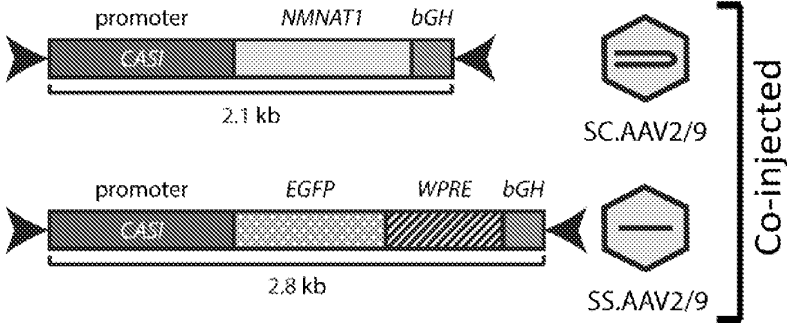
676-750 ATCAGAAGGGCCCTGAGCGGGGACAGTCCGATCAGGTACTTGGTCCCAGACCTTGTCAGAGTACATTGAAAAG
 C G A C AA C AGC TC C A T A

751-825 CACAACCTCTACAGCTCAGAGTCAGAGGATCGCAATGCAGGAGTGATCCTGGCCCTCTCCAGCGGAACACTGCA
 T TT G T AGT A CA G T G C T G A A

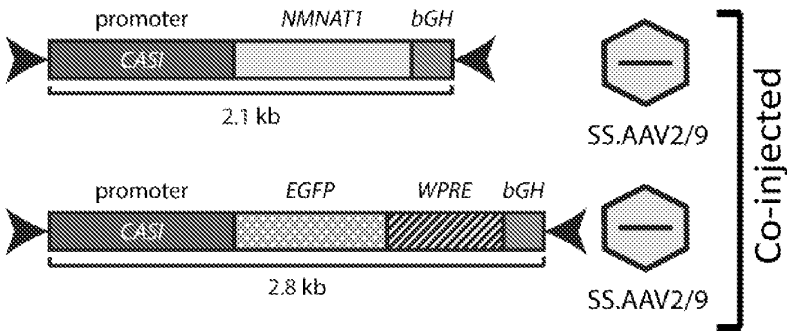
826-840 GAGGCCAAGACTTAG
 A T A

FIG. 1A

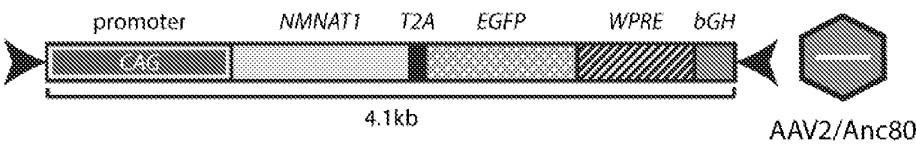
B Self-complementary AAV2/9 reagent (SC.AAV2/9)



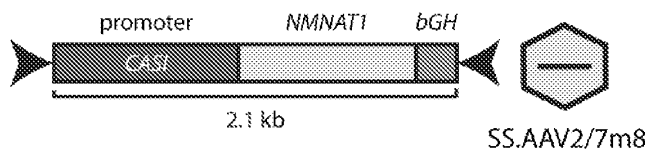
C Single-stranded AAV2/9 reagent (SS.AAV2/9)



D Single-stranded reagent (AAV2/Anc80)



E Single-stranded reagent (AAV2/7m8)



FIGs. 1B-E

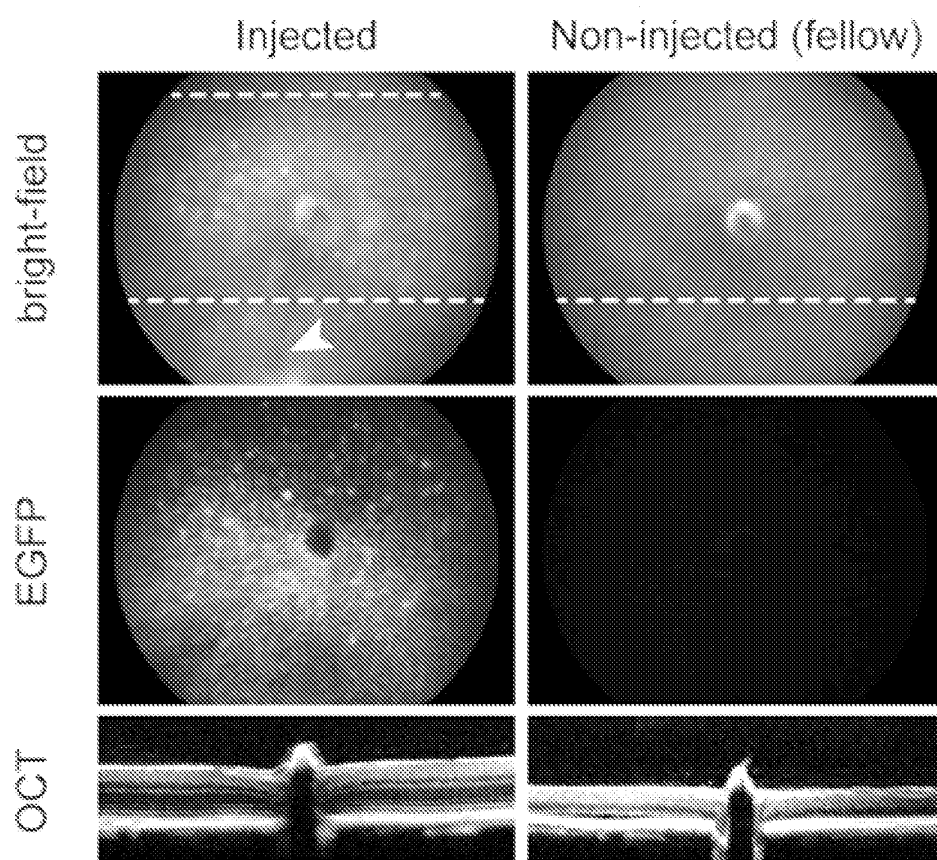


FIG. 2

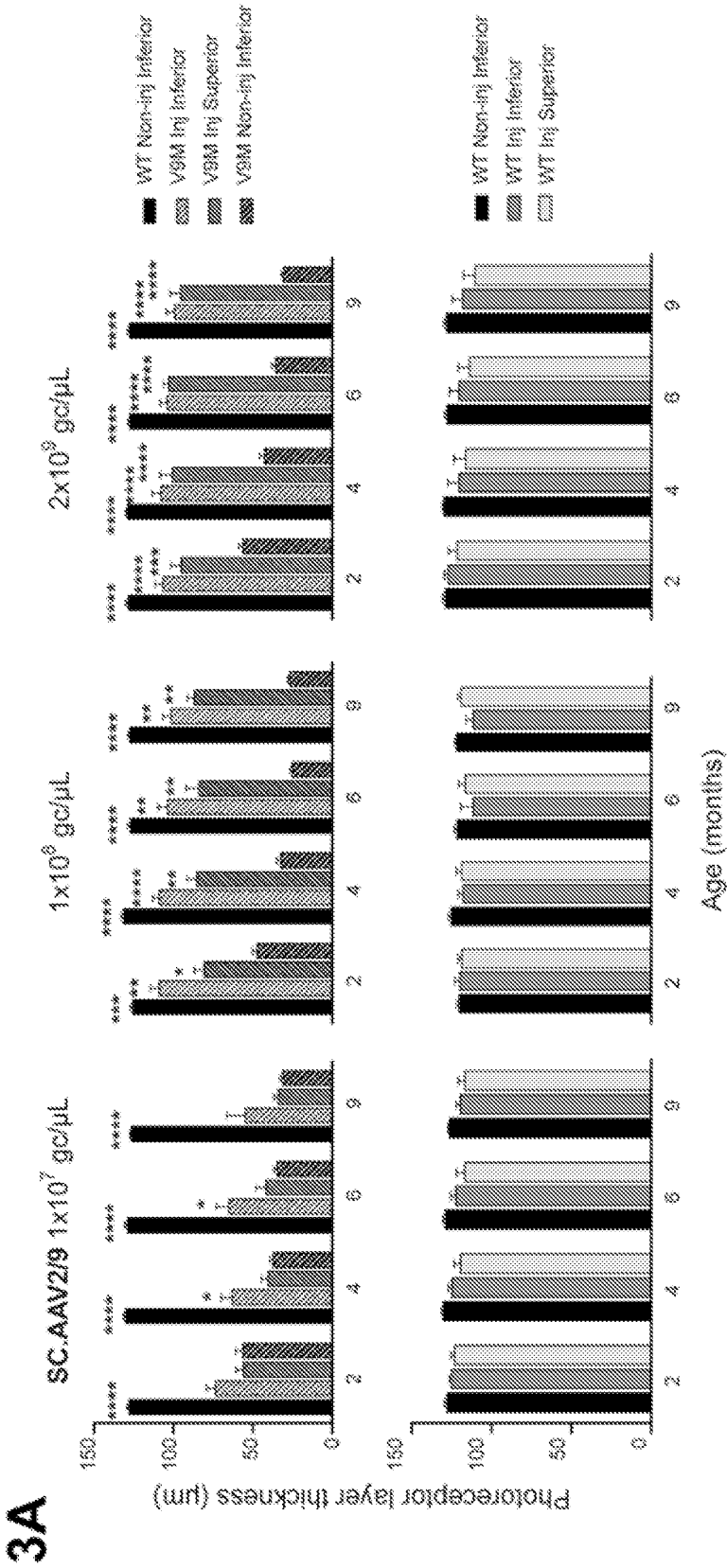
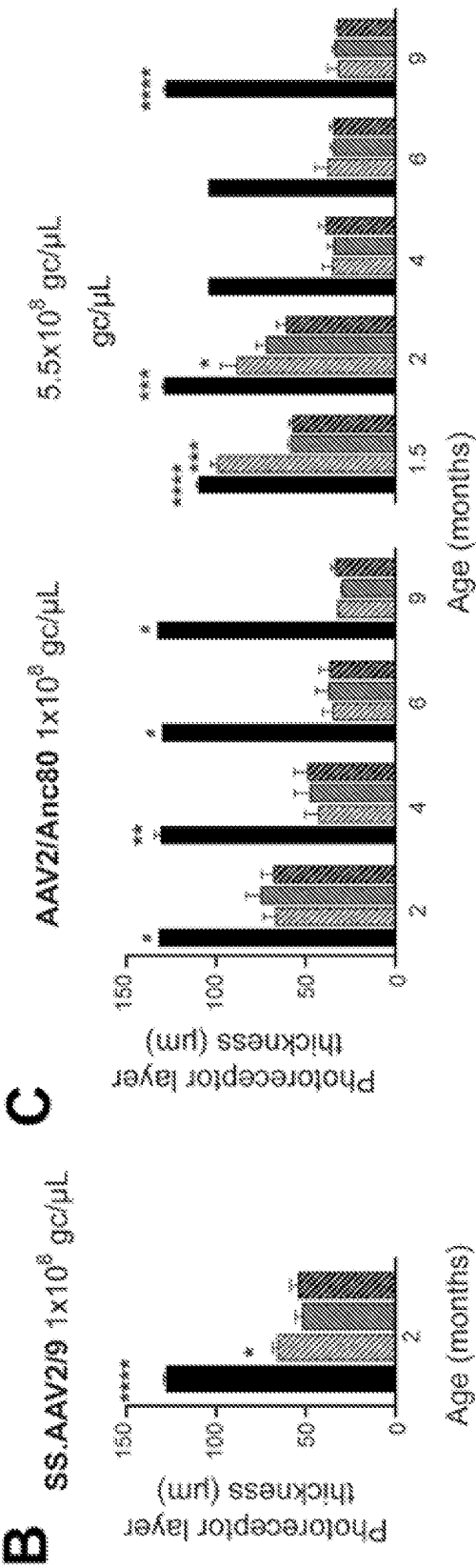


FIG. 3A



FIGs. 3B-C

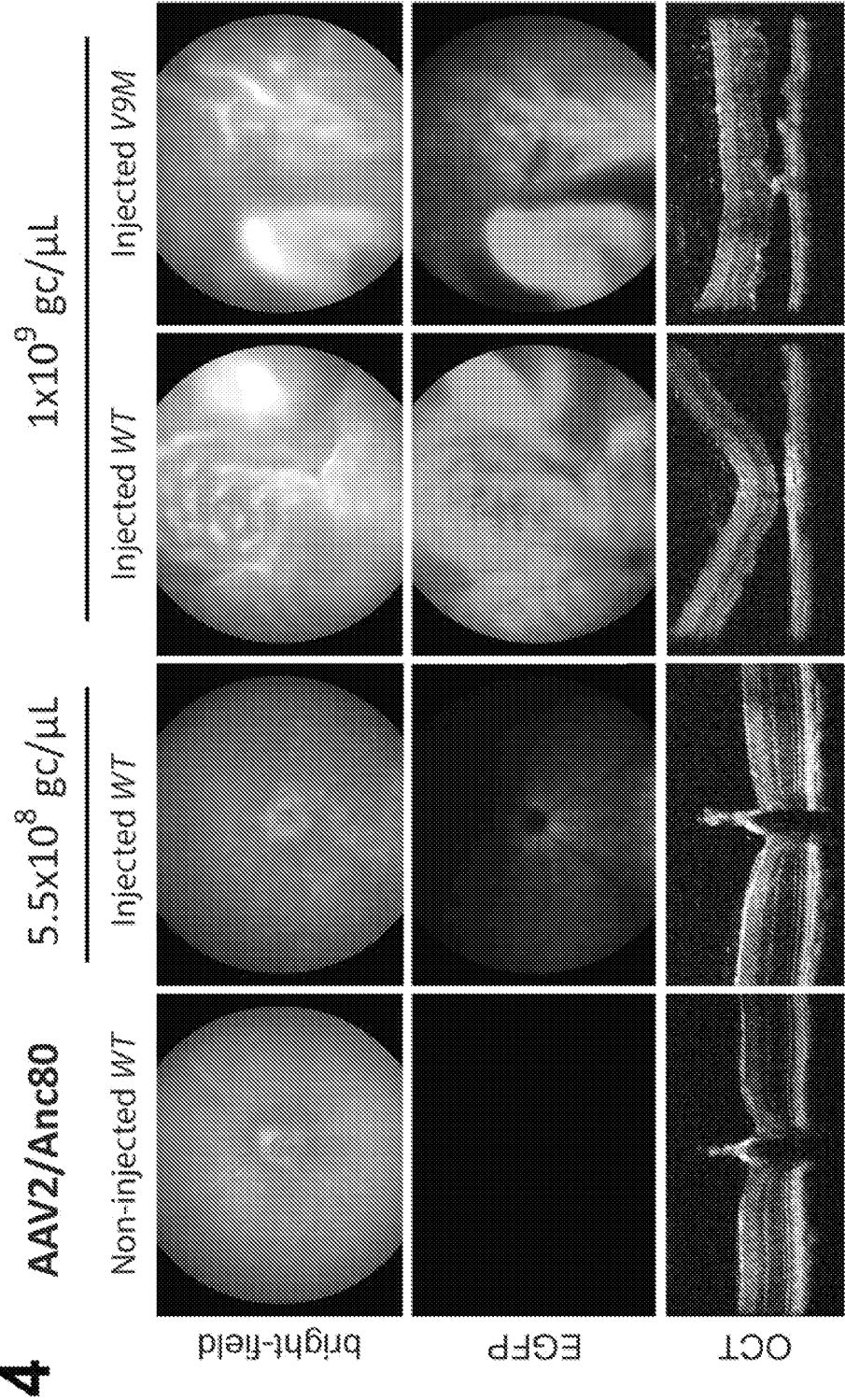


FIG. 4

5A

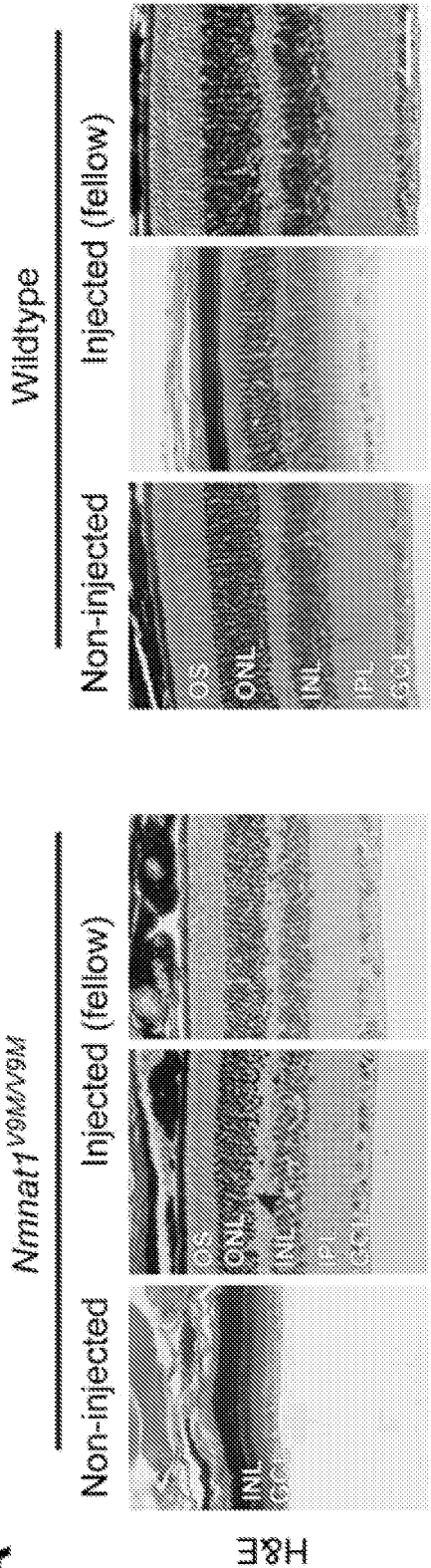


FIG. 5A

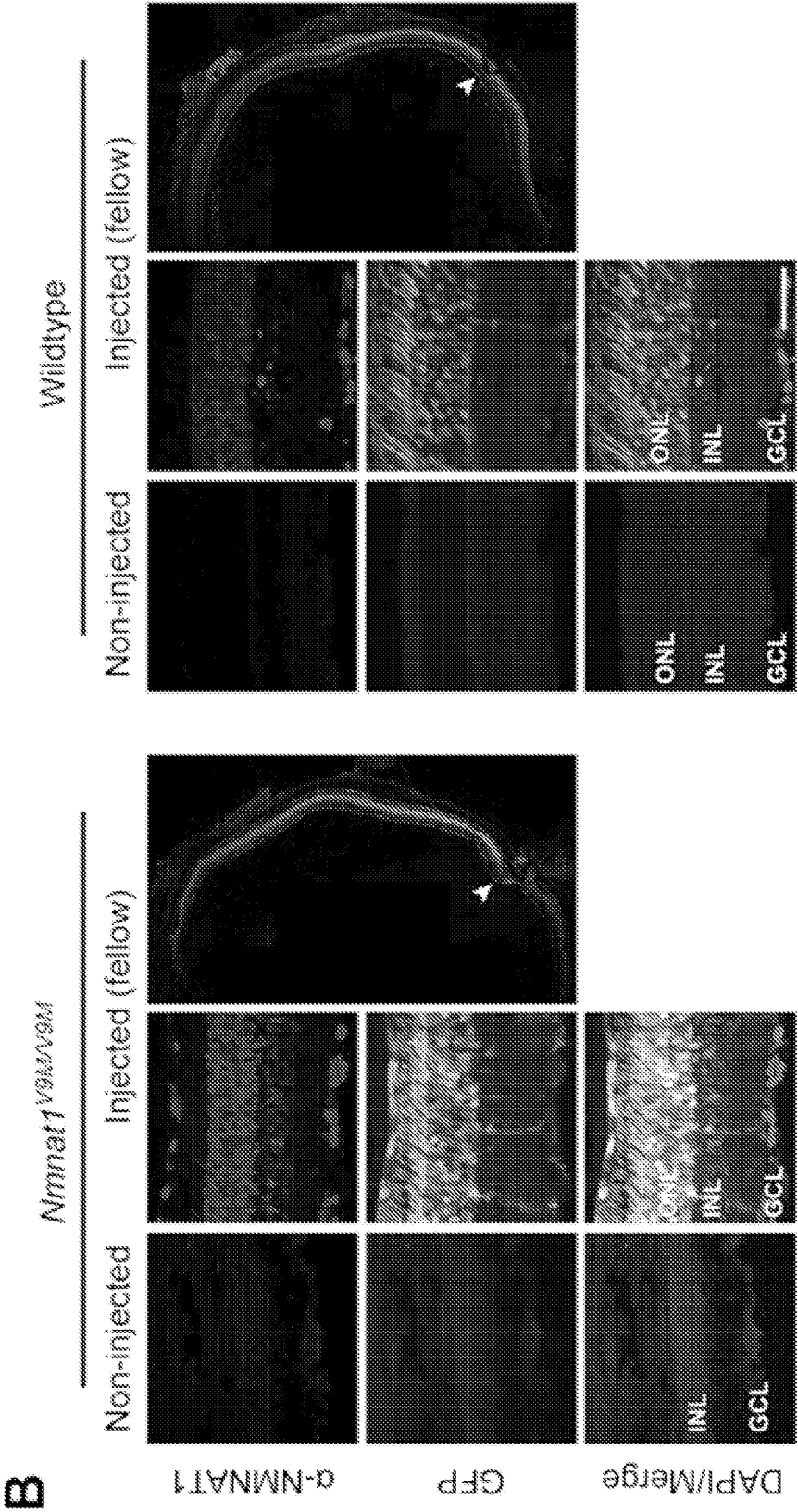


FIG. 5B

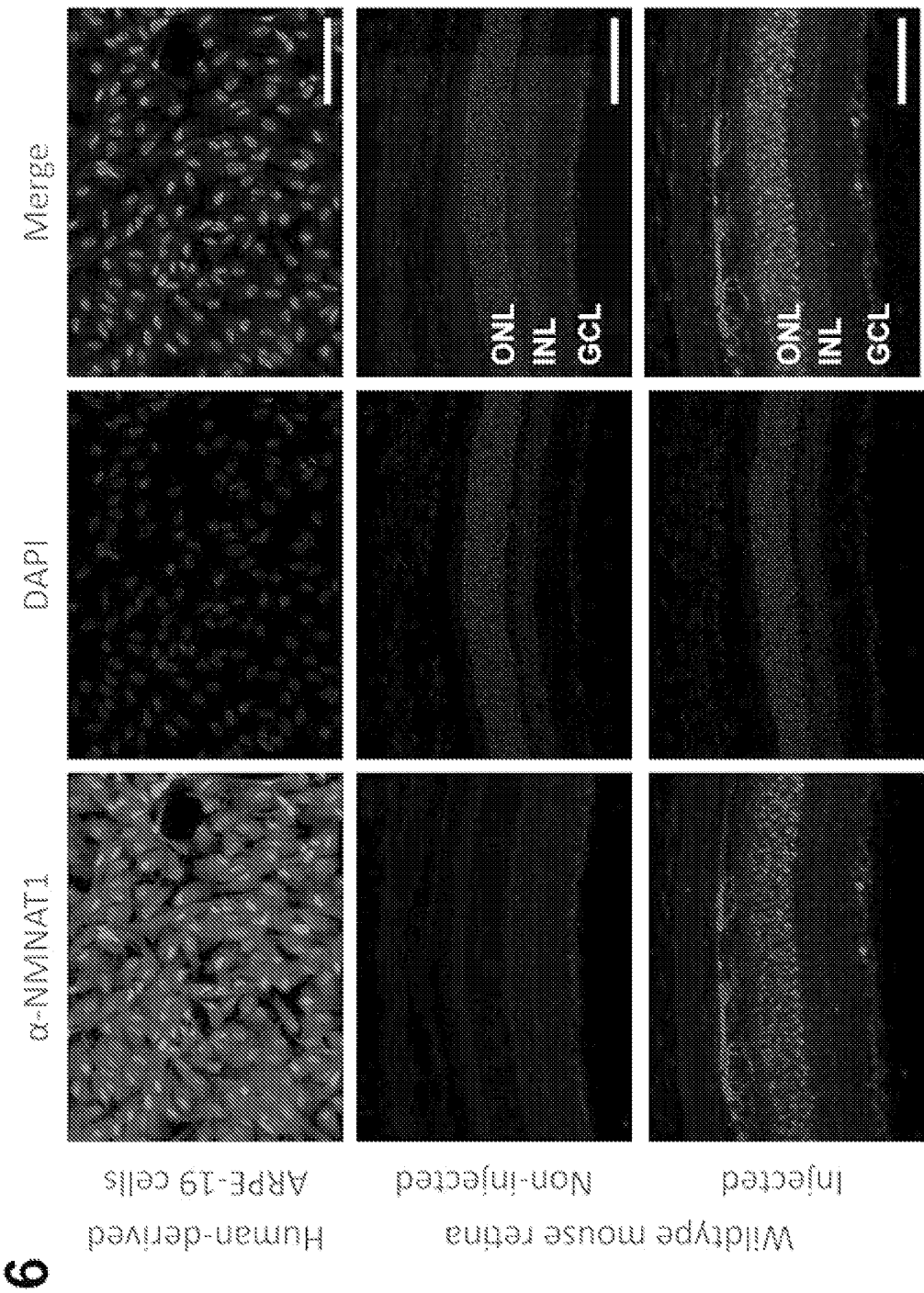


FIG. 6

7A

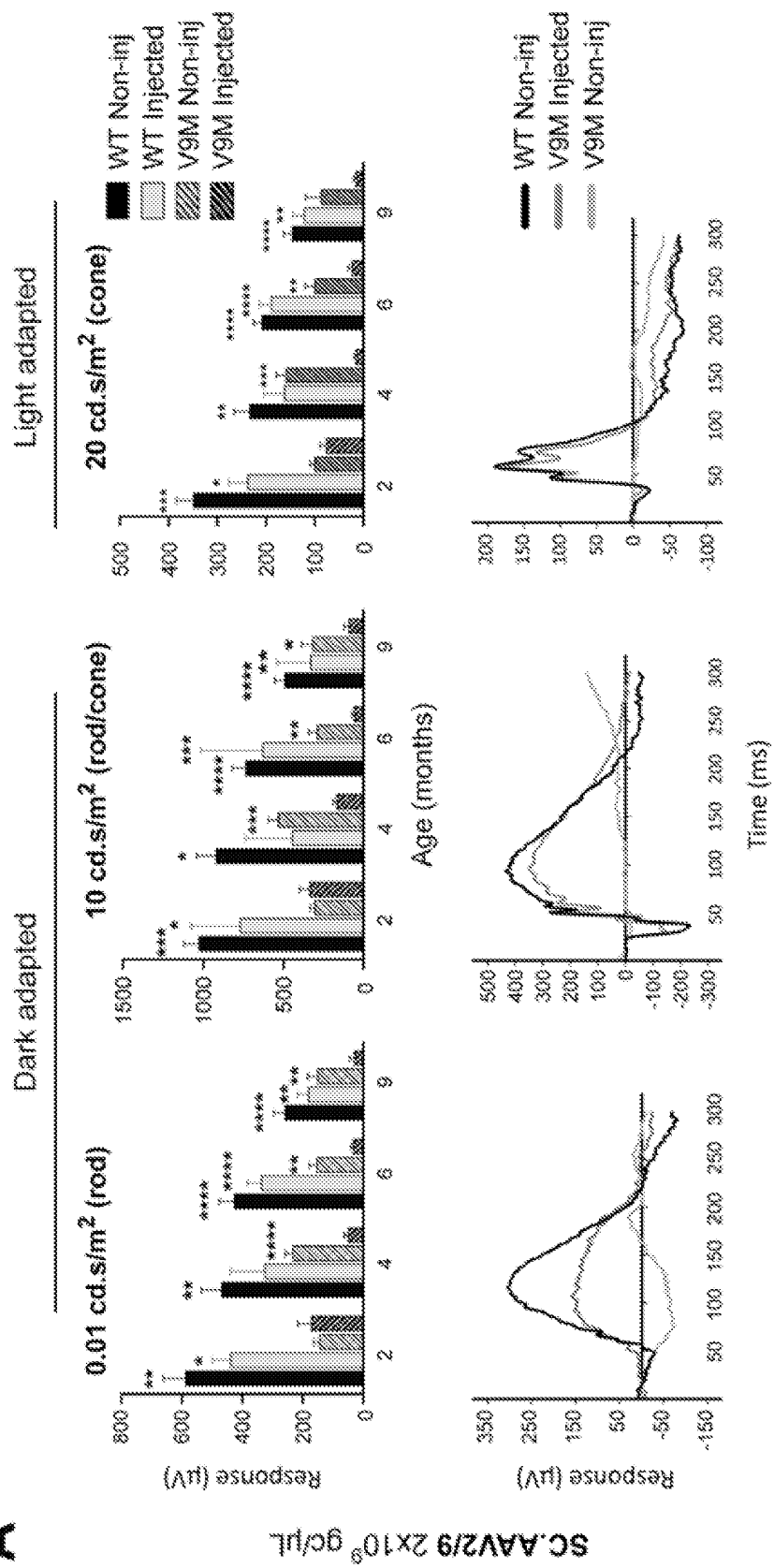


FIG. 7A

B

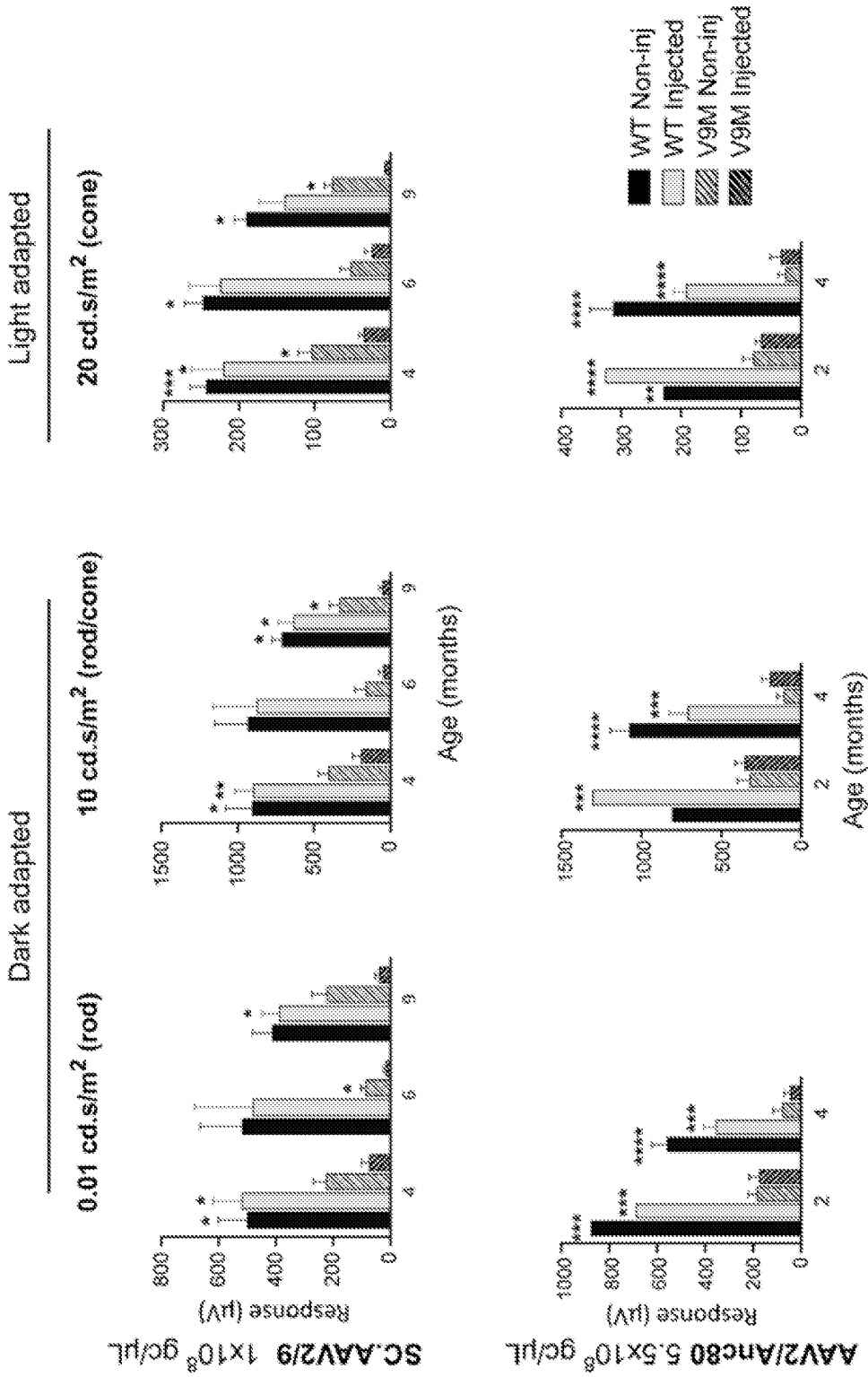
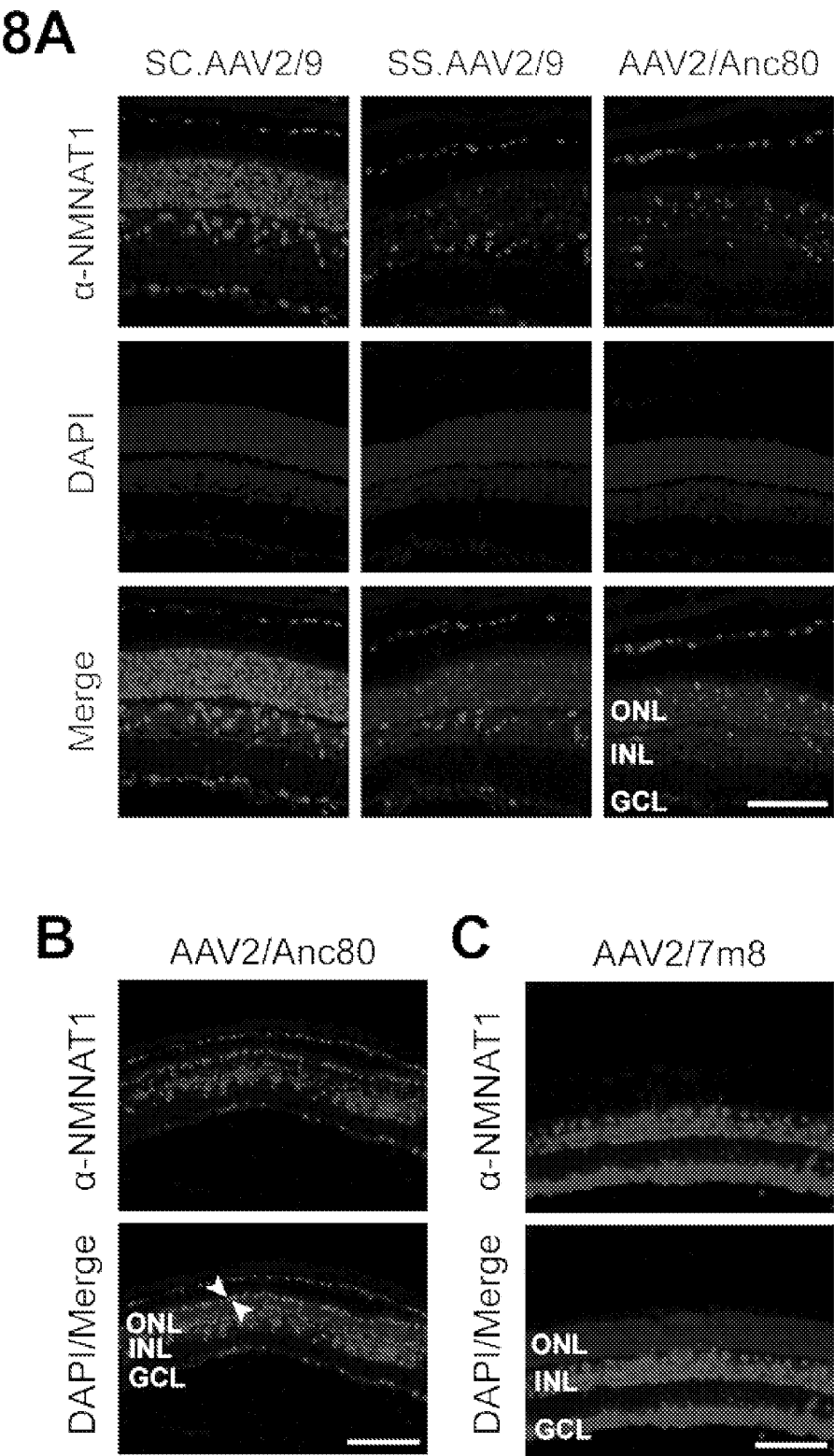


FIG. 7B



FIGs. 8A-C