WHAT IS CLAIMED IS:

1. Use of a therapeutically effective amount of an Adeno-associated virus (AAV) vector comprising a codon-optimized nucleic acid sequence encoding human NMNAT1, operably linked to a CASI promoter that drives expression in retinal cells, wherein the codon-optimized nucleic acid sequence encoding the human NMNAT1 is at least 98% identical to SEQ ID NO: 1, in the manufacture of a medicament for treating retinal degeneration caused by mutations in a nicotinamide mononucleotide adenylyltransferase 1 (*NMNAT1*) gene in a human subject.

2. The use of claim 1 wherein the vector further comprises a bovine growth hormone (bGH) polyadenylation signal sequence.

3. The use of claim 2, wherein the human NMNAT1 comprises the amino acid sequence of SEQ ID NO: 3.

4. The use of claims 1-3, wherein the vector is to be delivered via sub-retinal injection.

5. An Adeno-associated virus type 2 (AAV2) vector comprising a codon-optimized nucleic acid sequence encoding human NMNAT1, operably linked to a CASI promotor that drives expression in retinal cells, wherein the codon-optimized nucleic acid sequence encoding the human NMNATI is at least 98% identical to SEQ ID NO:1.

6. The vector of claim 5, wherein the vector further comprises a bovine growth hormone (bGH) polyadenylation signal sequence.

7. The vector of claim 5, wherein the human NMNAT1 comprises the amino acid sequence of SEQ ID NO:3.

8. A pharmaceutical composition comprising the vector of claims 5-7, formulated for delivery via sub-retinal injection.

9. The vector of any one of claims 5-7, wherein the vector comprises from 5´ to 3´: the CASI promoter, the codon-optimized nucleic acid sequence encoding the human NMNAT1, and the bGH polyadenylation signal sequence.

10. An Adeno-associated virus (AAV) vector comprising a codon-optimized nucleic acid sequence encoding human NMNAT1, operably linked to a CAG promoter that drives expression in retinal cells, wherein the codon-optimized nucleic acid sequence encoding the human NMNAT1 is at least 98% identical to SEQ ID NO:1.

11. An Adeno-associated virus (AAV) vector comprising a codon-optimized nucleic acid sequence encoding human NMNAT1, operably linked to a CMV promoter that drives expression in retinal cells, wherein the codon-optimized nucleic acid sequence encoding the human NMNAT1 is at least 98% identical to SEQ ID NO:1.

12. An Adeno-associated virus (AAV) vector comprising an Adeno-associated virus type 9 (AAV9) capsid and a codon-optimized nucleic acid sequence encoding human NMNAT1, wherein the codon-optimized nucleic acid sequence encoding the human NMNAT1 is at least 98% identical to SEQ ID NO:1.

13. The use of any one of claims 1-3, wherein the vector further comprises an Adeno-associated virus type 9 (AAV9) capsid.

14. The vector of any one of claims 5-7, further comprising an Adeno-associated virus type 9 (AAV9) capsid.

15. The vector of any one of claims 5-7 and 10-12, wherein the vector is a single-stranded or self-complementary vector.