CLAIMS

1. A vector comprising a nucleic acid molecule comprising a sequence encoding a non-mutant or functional CYP4V2 protein or a functional variant thereof having at least 96% sequence identity with any of SEQ ID NOs: 4-6 and 19-29 operably linked to at least one regulatory sequence, wherein the vector is a recombinant adeno-associated virus (rAAV) vector.

2. The vector of claim 1, wherein the rAAV vector comprises a VP1, VP2, or VP3 capsid protein selected from any serotype of AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAV10, AAV11, AAV12, or another naturally derived serotype or isolate or clade of AAV, or hybrids, variants or derivatives thereof, or

wherein the rAAV vector comprises a 5′ AAV inverted terminal repeat (ITR) or a 3′ AAV ITR selected from any one of AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAV10, AAV11, AAV12, or another naturally derived serotype or isolate or clade of AAV, or mutations, chimeras, variants or fusions thereof.

3. The vector of claim 1 or 2, wherein the rAAV vector is a chimeric AAV, a shuffled AAV, or a capsid-modified AAV, or

wherein the rAAV vector is a pseudotyped AAV, or

wherein the rAAV vector is a hybrid AAV.

4. The vector of any one of claims 1-3, wherein the rAAV vector comprises one or more mutations to one or more naturally derived AAV capsid proteins,
optionally wherein the one or more mutations to the AAV capsid protein comprise one or more amino acid residue Y-F, K-R, T-A, S-A or T-V substitutions.

5. The vector of any one of claims 1-4, wherein the rAAV vector is selected from the group consisting of AAV2/5, AAV2/8, AAV2/2, AAV2 (Y444F+Y500F+Y730F), AAV2/1, AAV2/9, AAV2/8(Y733F), AAV2/6, AAV2/4, AAV2/7, AAV5, AAV2,

AAV8, AAV1, AAV9, AAV6, AAV10, AAV3, AAV4, AAV7, AAV11, AAV12, Anc80, AAV 7m8, AAV-DJ, ShH10, AAV-PHP.B, rh10, and a hybrid, a derivative or variant thereof, or

wherein the rAAV vector is a single-stranded AAV vector or a self-complementary AAV (scAAV) vector.

6. The vector of any one of claims 1-5, wherein the nucleic acid molecule has at least 76% sequence identity with any of SEQ ID NOs: 1 – 3.

7. The vector of any one of claims 1-6, wherein the regulatory sequence comprises a promoter,

optionally wherein the promoter is a retinal pigment epithelium (RPE) cell-specific promoter, a photoreceptor-specific promoter, a retinal cell-specific promoter, a corneal cell-specific promoter, an ocular cell-specific promoter, a cell-specific promoter, a tissue-specific promoter, a constitutive promoter, a ubiquitous promoter, a regulated promoter, an inducible promoter, or a derivative or hybrid thereof, or

optionally wherein the promoter is selected from the group consisting of a CAG promoter (also known as CAGGS promoter, CB promoter or CBA promoter), a chicken beta actin promoter, a small CBA (smCBA) promoter, a CB^{SB} promoter, a CBh promoter, a beta-actin promoter, a human beta actin promoter, an elongation factor 1 alpha short (EFS) promoter, an elongation factor 1 alpha (EF-1 alpha or EF-1α) promoter, a cytomegalovirus (CMV) promoter, a PGK promoter, a UBC promoter, a GUSB promoter, a UCOE promoter, a VMD2 (vitelliform macular dystrophy 2; also known as BEST1) promoter, an RPE65 promoter, a GRK1 promoter, a human interphotoreceptor retinoid-binding protein proximal (IRBP) promoter, a 235 nt fragment of the hIRBP promoter, an RPGR proximal promoter, the red opsin promoter, a red-green opsin promoter, the blue opsin promoter, the mouse opsin promoter, a rhodopsin (Rho) promoter, a beta phosphodiesterase (PDE) promoter, a retinitis pigmentosa (RP1) promoter, the NXNL2/NXNL1 promoter, a retinal degeneration slow/peripherin 2 (Rds/perphZ) promoter, a IRBP/GNAT2 promoter (hIRBP enhancer fused to cone

transducin alpha promoter), an Rds (retinal degeneration slow) promoter, an hPDE6b promoter, a VEcad promoter (VE-cadherin/Cadherin 5 (CDH5)/CD144 promoter), a calcium-sensitive promoter, an NFAT promoter, a zinc-inducible sheep metalioihionine (MX) promoter, a dexamethasone (Dex)-inducible mouse mammary tumor virus (MMTV) promoter, a T7 polymerase promoter system, an ecdysone insect promoter, a tetracycline-repressible system, a tetracycline-inducible system, an RU486-inducible system, and a rapamycin-inducible system, a hybrid, a variant and a derivative thereof.

8. The vector of any one of claims 1-7, wherein the regulatory sequence comprises a Kozak sequence, or

wherein the regulatory sequence comprises an enhancer,

optionally wherein the enhancer comprises a post-transcriptional regulatory element (PRE), an internal ribosome entry site (IRES), an intron regulatory sequence, a mini-intron splice donor/splice acceptor (SD-SA), or a constitutive transport element (CTE), or

optionally wherein the enhancer is selected from the group consisting of a woodchuck hepatitis virus post-transcriptional regulatory element (WPRE) enhancer, an Hepatitis B Virus post-transcriptional regulatory element (HPRE or HBVPRE) enhancer, an IRBP enhancer, a Mason-Pfizer Monkey Virus CTE enhancer, an Avian Leukemia Virus CTE enhancer, a cytomegalovirus (CMV) immediate early enhancer, and an SV40 enhancer.

9. The vector of any one of claims 1-8, wherein the regulatory sequence comprises a polyadenylation (polyA) signal,

optionally wherein the polyA signal is selected from the group consisting of a bovine growth hormone polyadenylation signal (bGH polyA), a small polyA signal (SPA), a human growth hormone polyadenylation signal (hGH polyA), an SV40 polyA signal, an SV40 early polyA signal, an SV40 late polyA signal, a derivative and a hybrid thereof.

10. The vector of any one of claims 1-9, wherein the regulatory sequence comprises an upstream enhancer (USE) operably linked to a polyA signal,

optionally wherein the USE is selected from the group consisting of SV40 late 2xUSE, HIV-1 (Human immunodeficiency virus 1) USE, GHV (Ground squirrel hepatitis virus) USE, Adenovirus (L3) USE, hTHGB (Human prothrombin) USE, and an hC2 (Human C2 complement gene) USE.

11. The vector of any one of claims 1-10, where in the regulatory sequence comprises an intron sequence, a UTR sequence, a splice site sequence, an upstream regulatory domain sequence, a response element sequence, an inducible element sequence, an origin of replication sequence, an internal ribosome entry sites (IRES) sequence, a transcription initiation sequence, a termination sequence, an RNA processing sequence, a junction sequence, or a linker sequence, or

wherein the regulatory sequence is heterologous or homologous relative to the CYP4V2 coding sequence whose expression said regulatory sequence regulates, or

wherein the regulatory sequence comprises a tissue-specific or cell-specific microRNA (miRNA) target sequence.

12. The vector of any one of claims 1-11, wherein any part of the vector shares at least 43% sequence identity with any of the CYP4V2 expression cassette sequences in SEQ ID NOs: 60 to 64 or at least 43% sequence identity with any of the following sequences: nucleotide (nt) 237 – nt 3579 of SEQ ID NO 60; nt 166 – nt 3515 of SEQ ID NO 61; nt 166 – nt 3515 of SEQ ID NO 62; nt 166 – nt 3515 of SEQ ID NO 63; or nt 130 – nt 2097 of SEQ ID NO 64.

13. The vector of any one of claims 1-12, wherein the vector is formulated with a pharmaceutically acceptable carrier and additional components suitable for a specific route of administration.

14. A cell comprising the vector of any one of claims 1-13,

optionally wherein the cell is a mammalian cell or an insect cell,

optionally wherein the cell is an HEK293 cell,

optionally wherein the cell is a retinal pigment epithelial (RPE) cell, a photoreceptor cell, a photoreceptor progenitor cell, a choroidal cell, a retinal cell, an induced pluripotent stem (iPS) cell, or a stem cell, of or derived from a human subject suffering from Bietti's Crystalline Dystrophy (BCD, a/k/a Bietti Crystalline Corneoretinal Dystrophy, Bietti Crystalline Retinopathy, Bietti's Retinal Dystrophy), or retinitis pigmentosa (RP) or inherited retinal degeneration (IRD) with CYP4V2 mutations.

15. Use of the cell of claim 14 in the manufacture of a medicament for treating, arresting or preventing Bietti's Crystalline Dystrophy (BCD, a/k/a Bietti Crystalline Corneoretinal Dystrophy, Bietti Crystalline Retinopathy, Bietti's Retinal Dystrophy), or retinitis pigmentosa (RP) or inherited retinal degeneration (IRD) with CYP4V2 mutations in a human subject in need thereof, wherein a therapeutically effective amount of the cell is to be delivered to the human subject's eye.

16. Use of the vector of any one of claims 1-13 in the manufacture of a medicament for treating, arresting or slowing progression of, rescuing or ameliorating the dysfunction, function loss, dystrophy, disorder, degeneration, atrophy or preventing the death of an ocular cell of a human subject who has Bietti's Crystalline Dystrophy (BCD, a/k/a Bietti Crystalline Corneoretinal Dystrophy, Bietti Crystalline Retinopathy, Bietti's Retinal Dystrophy), or retinitis pigmentosa (RP) or inherited retinal degeneration (IRD) with CYP4V2 mutations, wherein a therapeutically effective amount of the vector is to be delivered to the ocular cell,

optionally wherein the ocular cell is a retina cell, a retinal pigment epithelial (RPE) cell, a photoreceptor cell (rod or cone), a choroidal epithelial cell, a corneal epithelium cell, a choroidal cell, a corneal cell, a retina bipolar cell, a photoreceptor progenitor cell, a ganglion cell, or an optic-nerve cell.

17. Use of the vector of any one of claims 1-13 in the manufacture of a medicament for treating, arresting or preventing Bietti's Crystalline Dystrophy (BCD, a/k/a Bietti Crystalline Corneoretinal Dystrophy, Bietti Crystalline Retinopathy, Bietti's Retinal Dystrophy), or retinitis pigmentosa (RP) or inherited retinal degeneration (IRD) with CYP4V2 mutations in a human subject in need thereof, wherein a therapeutically effective amount of the vector is to be delivered to one or more ocular cells of the human subject,

wherein the one or more ocular cells are selected from retinal pigment epithelial (RPE) cells, one or more choroidal cells, and one or more photoreceptor cells of the human subject.

18. Use of the vector of any one of claims 1-13 in the manufacture of a medicament for treating, arresting, or preventing a human subject’s disease which is associated with the atrophy, dystrophy, dysfunction, degeneration or death of an ocular cell, wherein a therapeutically effective amount of the vector is to be delivered to the ocular cell of the human subject,

wherein the ocular cell is an RPE cell.

19. Use of the vector of any one of claims 1-13 in the manufacture of a medicament for treating, arresting or preventing a human subject’s disease which is associated with the atrophy, dystrophy, dysfunction, degeneration or death of an ocular cell, wherein a therapeutically effective amount of RPE cell, photoreceptor progenitor cell or stem cell is to be delivered to the human subject's eye,

wherein said RPE cell, photoreceptor progenitor cell or stem cell comprises the vector.

20. The cell of claim 14 or the vector of any one of claims 1-13, for use in therapy.