

## Supplemental Table 1

### Inherited retinal disease genes:

*ABCA4, ABCC6, ABHD12, ACBD5, ADAM9, ADAMTS18, AHI1, AIPL1, ALMS1, ARL13B, ARL2BP, ARL2BP, ARL6, ATXN7, BBIP1, BBIP1, BBS1, BBS10, BBS12, BBS2, BBS4, BBS5, BBS7, BBS9, BEST1, C1QTNF5, C21orf2, C2ORF71, C5orf42, C8orf37, CA4, CABP4, CACNA1F, CACNA2D4, CAPN5, CC2D2A, CDH23, CDH3, CDHR1, CEP164, CEP290, CEP41, CEP83, CERKL, CHM, CIB2, CLN2, CLN3, CLN5, CLN6, CLN8, CLRN1, CNGA1, CNGA3, CNGB1, CNGB3, CNNM4, COL11A1, COL2A1, COL9A1, CRB1, CRX, CSPP1, CYP4V2, DFNB31, DHDDS, DHX38, DTHD1, EFEMP1, ELOVL4, EMC1, ERCC6, EYS, FAM161A, FLVCR1, FSCN2, FZD4, GDF6, GNAT1, GNAT2, GNB1, GNPTG, GPR125, GPR143, GPR179, GPR98, GRK1, GRM6, GRN, GUCA1A, GUCA1B, GUCY2D, HARS, IDH3B, IFT122, IFT140, IFT172, IFT27, IFT43, IFT80, IFT88, IKBKG, IMPDH1, IMPG1, IMPG2, INPP5E, INVS, IQCB1, ITM2B, JAG1, KCNJ13, KCNV2, KCTD7, KIAA1549, KIF11, KIZ, KLHL7, LCA5, LRAT, LRIT3, LRP5, LZTFL1, MAK, MERTK, MFN2, MFRP, MFSD8, MKKS, MKS1, MTPP, MVK, MYO7A, NDP, NEK2, NMNAT1, NPHP1, NPHP3, NPHP4, NR2E3, NRL, NUB1, NYX, OAT, OCA2, OFD1, OPA1, OPA3, OPN1LW, OPN1MW, OPN1SW, OTX2, PANK2, PAX2, PCDH15, PCYT1A, PDE6A, PDE6B, PDE6C, PDE6G, PDE6H, PDZD7, PEX1, PEX10, PEX14, PEX16, PEX19, PEX2, PEX5, PEX6, PEX7, PGK1, PHYH, PITPNM3, PLA2G5, POC1B, PPT1, PRCD, PROM1, PRPF3, PRPF31, PRPF4, PRPF6, PRPF8, PRPH2, RAB28, RAX2, RBP3, RBP4, RD3, RDH12, RDH5, RGR, RGS9, RGS9BP, RHO, RIMS1, RLBP1, ROM1, RP1, RP1L1, RP2, RP9, RPE65, RPGR, RPGRIP1, RPGRIP1L, RS1, SAG, SDCCAG8, SEMA4A, SLC24A1, SLC45A2, SLC4A5, SLC7A14, SNRNP200, SPATA7, TEAD1, TIMM8A, TIMP3, TMEM126A, TMEM231, TMEM237, TMEM67, TOPORS, TPP1, TREX1, TRIM32, TRPM1, TSPAN12, TTC21B, TTC8, TTLL5, TTPA, TUB, TULP1, TYR, TYRP1, UNC119, USH1C, USH1G, USH2A, USH3A, VCAN, VPS13B, WDPCP, WDR19, WDR34, WDR35, WFS1, ZNF423, ZNF513*

### Human retinal gene therapy clinical trials:

Phase 1, 1/2, and 3 *RPE65*-Leber congenital amaurosis  
Phase 1/2 *ABCA4*-Stargardt disease  
Phase 1/2 *CHM*-choroideremia  
Phase 1/2 *RS1*-X-linked retinoschisis  
Phase 1/2 *MYO7A*-Usher syndrome  
Phase 1/2 *CNGA3*-Achromatopsia  
Phase 1/2 *CNGB3*-Achromatopsia  
Phase 1 *MERTK*-retinitis pigmentosa  
Phase 1 *ND4*-Leber's Hereditary Optic Neuropathy

### Preclinical animal retinal gene-therapy studies:

*ABCA4*-Stargardt disease  
*AIPL1*-cone-rod dystrophy and Leber congenital amaurosis  
*BBS1*-Bardet Biedl syndrome and retinitis pigmentosa  
*BEST1*-macular dystrophy  
*CEP290*-retinal dystrophy  
*CDH3*-retinal dystrophy  
*CHM*-choroideremia  
*CNGA3*-Achromatopsia  
*CNGB3*-Achromatopsia  
*CRB1*-retinal dystrophy  
*GUCY2D*-Leber congenital amaurosis  
*MERTK*-retinitis pigmentosa  
*MRFP*-retinitis pigmentosa  
*MYO7A*-Usher syndrome  
*ND4*-Leber's Hereditary Optic Neuropathy  
*NR2E3*-retinitis pigmentosa and enhanced S-cone syndrome  
*PDE6β*-retinitis pigmentosa  
*PRPH2*-retinal dystrophy  
*RD3*-Leber congenital amaurosis  
*RHO*-retinitis pigmentosa  
*RLBP1*-retinitis pigmentosa  
*RP1*-retinitis pigmentosa  
*RPE65*-Leber congenital amaurosis  
*RPGR*-X-linked retinitis pigmentosa  
*RPGRIP1*-Leber congenital amaurosis and cone-rod dystrophy  
*RS1*-X-linked retinoschisis  
*SPATA7*-Leber congenital amaurosis and retinitis pigmentosa