

Supplemental Table 1. Representative AAV dosage, titer and MOI calculation for iPSC, RPE, human and rat cortical cells.

Supplemental Table 2. Relative fluorescence intensity (R.F.U) per cell at 96h at MOI (1E6 vg/cell)

Supplementary Figure 1. Stable GFP transgene expression in RPE. (A) The AAV-eGFP expression in RPE at d4 (96h) and d18 for a dosage of 1E6 vg/cell show similar expression in longer term cultures. There is no statistically significance difference between day 4 and day 18 in any of the AAV.

Supplementary Figure 2. RPE and Cortical Neuron Characterization. (A) Flow cytometric analysis of intracellular MITF protein expression. Greater than 95% of RPE express MITF. Plot showing MITF positive cells (blue) relative to mouse isotype control antibody (red). (B-C) Immunophenotyping of human and rat cortical neurons and astrocytes showing neuron-specific class III beta-tubulin (TUJ1; green) and glial fibrillary acidic protein (GFAP; red), respectively. Images were capture at 20x magnification (human iPSC-cortical) and 10x magnification (rat cortical).

Supplemental Table 1: Representative AAV dosage, titer and MOI calculation for iPSC, RPE, human and rat cortical cells.

| Serotypes | Lot Number | Titer | iPSC | | | IPSC-RPE | | | Ex Vivo Rat Corticals | | | Human iPSC-derived Corticals | | |
|-----------------------|------------|----------|---------------------------|-------|-------|---------------------------|-------|-------|---------------------------|-------|-------|------------------------------|-------|-------|
| | | | MOI (vector genomes/cell) | | | MOI (vector genomes/cell) | | | MOI (vector genomes/cell) | | | MOI (vector genomes/cell) | | |
| | | | 1E+04 | 1E+05 | 1E+06 | 1E+04 | 1E+05 | 1E+06 | 1E+04 | 1E+05 | 1E+06 | 1E+04 | 1E+05 | 1E+06 |
| AAV1-CMV/CBA-eGFP | CT 290 | 8.95E+12 | 2.3 | 2.3 | 2.3 | 8.8 | 8.8 | 8.8 | 22.3 | 22.3 | 22.3 | 11.2 | 11.2 | 11.2 |
| AAV2-CMV/CBA-eGFP | CT 176 | 3.28E+12 | 6.4 | 6.4 | 6.4 | 24.0 | 24.0 | 24.0 | 61.0 | 61.0 | 61.0 | 30.5 | 30.5 | 30.5 |
| AAV3-CMV/CB-eGFP | SAB-589 | 1.59E+13 | 1.3 | 1.3 | 1.3 | 5.0 | 5.0 | 5.0 | 12.6 | 12.6 | 12.6 | 6.3 | 6.3 | 6.3 |
| AAV4-CMV/CBA-eGFP | CT 318 | 9.58E+12 | 2.2 | 2.2 | 2.2 | 8.2 | 8.2 | 8.2 | 20.9 | 20.9 | 20.9 | 10.4 | 10.4 | 10.4 |
| AAV5-CMV/CBA-eGFP | CT 73 | 1.18E+13 | 1.8 | 1.8 | 1.8 | 6.7 | 6.7 | 6.7 | 16.9 | 16.9 | 16.9 | 8.5 | 8.5 | 8.5 |
| AAV6-CMV/CBA-eGFP | CT 291 | 9.51E+12 | 2.2 | 2.2 | 2.2 | 8.3 | 8.3 | 8.3 | 21.0 | 21.0 | 21.0 | 10.5 | 10.5 | 10.5 |
| AAV7-CMV/CBA-eGFP | CT 319 | 1.6E+13 | 1.3 | 1.3 | 1.3 | 4.9 | 4.9 | 4.9 | 12.5 | 12.5 | 12.5 | 6.3 | 6.3 | 6.3 |
| AAV7m8-CMV/CBA-eGFP | SAB-608 | 6.51E+12 | 3.2 | 3.2 | 3.2 | 12.1 | 12.1 | 12.1 | 30.7 | 30.7 | 30.7 | 15.4 | 15.4 | 15.4 |
| AAV8-CMV/CBA-eGFP | CT 118 | 1.11E+13 | 1.9 | 1.9 | 1.9 | 7.1 | 7.1 | 7.1 | 18.0 | 18.0 | 18.0 | 9.0 | 9.0 | 9.0 |
| AAV8b-CMV/CBA-eGFP | CT 320 | 1.16E+13 | 1.8 | 1.8 | 1.8 | 6.8 | 6.8 | 6.8 | 17.2 | 17.2 | 17.2 | 8.6 | 8.6 | 8.6 |
| AAV9-CMV/CBA-eGFP | CT 292 | 7.88E+12 | 2.7 | 2.7 | 2.7 | 10.0 | 10.0 | 10.0 | 25.4 | 25.4 | 25.4 | 12.7 | 12.7 | 12.7 |
| Dilution ratio | | | 1:100 | 1:10 | Stock | 1:100 | 1:10 | Stock | 1:1000 | 1:100 | 1:10 | 1:100 | 1:10 | Stock |

| Cell Type | Cell Number |
|-----------------------|-------------|
| iPSCs | 21,000 |
| iPSC-RPEs | 78,750 |
| Ex Vivo Rat Corticals | 20,000 |
| Human iPSC-Corticals | 100,000 |

Supplemental Table 2. Relative fluorescence intensity (R.F.U) per cell at 96h at MOI (1E6 vg/cell)

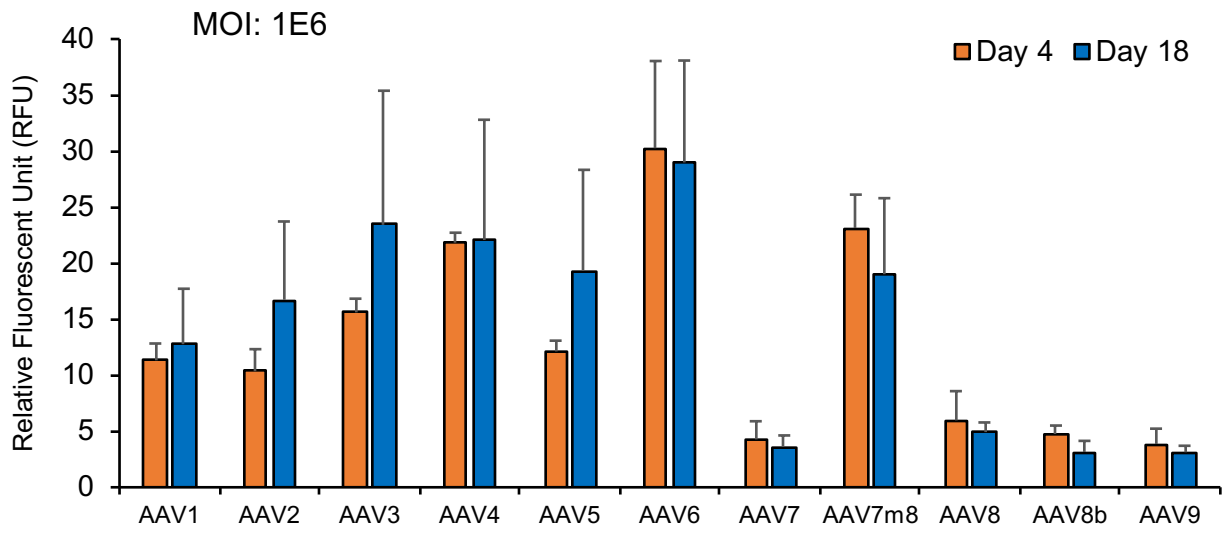
| iPSC | | | iPSC-RPE | | | Human iPSC-Cortical Neurons | | | Ex Vivo Rat Cortical Neurons | | |
|---------------|-------------------------|--------|---------------|-------------------------|--------|-----------------------------|-------------------------|--------|------------------------------|-------------------------|--------|
| AAV type | Mean [§] R.F.U | SEM | AAV type | Mean [§] R.F.U | SEM | AAV type | Mean [§] R.F.U | SEM | AAV type | Mean [§] R.F.U | SEM |
| AAV3 | 11.01 ^a | ± 1.55 | AAV6 | 22.86 ^a | ± 4.23 | AAV8b | 4.06 ^a | ± 0.46 | AAV6 | 16.79 ^a | ± 3.39 |
| AAV7m8 | 10.19 ^a | ± 1.35 | AAV7m8 | 17.54 ^{ab} | ± 2.91 | AAV7m8 | 3.87 ^{ab} | ± 0.65 | AAV7m8 | 13.15 ^{ab} | ± 2.23 |
| AAV6 | 9.20 ^a | ± 0.91 | AAV4 | 16.33 ^{abc} | ± 2.76 | AAV8 | 3.09 ^{abc} | ± 0.38 | AAV7 | 9.72 ^{abc} | ± 3.55 |
| AAV2 | 7.77 ^a | ± 0.78 | AAV3 | 11.86 ^{bcd} | ± 1.90 | AAV6 | 2.81 ^{abcd} | ± 0.41 | AAV1 | 7.42 ^{bcd} | ± 1.18 |
| AAV8 | 3.18 ^b | ± 0.40 | AAV5 | 8.82 ^{bcd} | ± 1.67 | AAV3 | 2.08 ^{bcde} | ± 0.37 | AAV8b | 7.14 ^{bcd} | ± 1.42 |
| AAV1 | 2.85 ^b | ± 0.36 | AAV1 | 8.43 ^{bcd} | ± 1.51 | AAV1 | 2.06 ^{bcde} | ± 0.48 | AAV4 | 6.21 ^{bcd} | ± 2.71 |
| AAV9 | 2.59 ^b | ± 0.34 | AAV2 | 7.83 ^{cd} | ± 1.37 | AAV9 | 1.93 ^{cde} | ± 0.35 | AAV8 | 5.33 ^{bcd} | ± 0.77 |
| AAV8b | 2.30 ^b | ± 0.30 | AAV8 | 4.68 ^d | ± 0.95 | AAV7 | 1.65 ^{cdef} | ± 0.23 | AAV9 | 2.82 ^{cd} | ± 0.48 |
| AAV4 | 1.43 ^b | ± 0.42 | AAV8b | 3.82 ^d | ± 0.51 | AAV2 | 1.15 ^{def} | ± 0.35 | AAV5 | 0.73 ^{cd} | ± 0.64 |
| AAV7 | 1.28 ^b | ± 0.30 | AAV7 | 3.66 ^d | ± 0.53 | AAV4 | 0.78 ^{ef} | ± 0.29 | AAV3 | 0.40 ^d | ± 0.84 |
| AAV5 | 0.52 ^b | ± 0.18 | AAV9 | 3.04 ^d | ± 0.55 | AAV5 | 0.06 ^f | ± 0.22 | AAV2 | 0.00 ^d | ± 0.75 |
| Pr > F(Model) | < 0.0001 [£] | | Pr > F(Model) | < 0.0001 [£] | | Pr > F(Model) | < 0.0001 [£] | | Pr > F(Model) | < 0.0001 [£] | |

*SEM: Standard error of mean

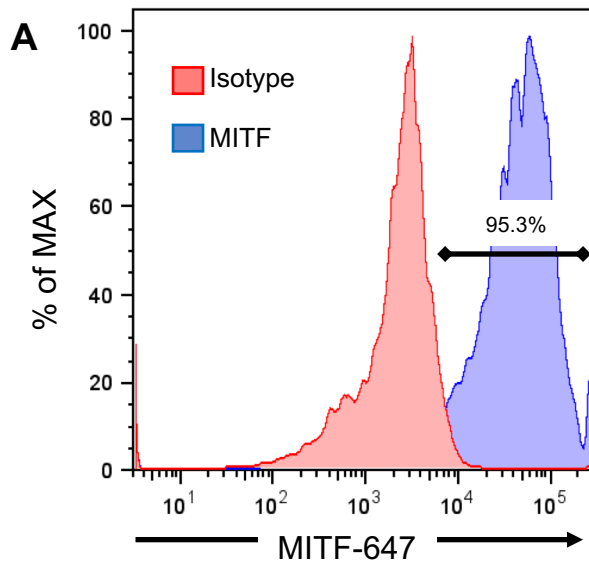
[£]For comparisons across 11 AAV groups to determine whether there are any statistically significant difference among them using one-way analysis of variance.

[§] The means noted with same superscript (a, b, c, d, e, f) indicate there are no statistically significant difference among them, but there are statistically significant difference among means noted with different superscript, after correction for multiple comparisons using Turkey method.

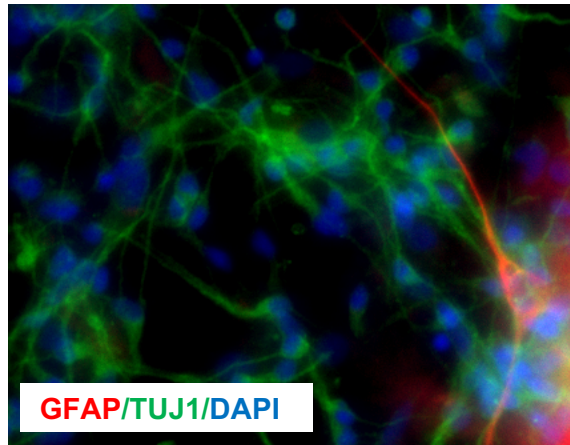
Supplemental Figure 1.



Supplemental Figure 2.



B Human iPSC-Derived
Cortical neurons



C Rat
Cortical neurons

