

Supplementary Table S1. Ophthalmic examination findings of vehicle-injected eyes

| <i>Finding</i> | <i>Predose</i> | <i>Study Day 0</i> | <i>Study Week 1</i> | <i>Study Week 2</i> | <i>Study Week 6</i> | <i>Study Week 10</i> | <i>Study Week 15</i> | <i>Study Week 20</i> |
|--|----------------|--------------------|---------------------|---------------------|---------------------|----------------------|----------------------|----------------------|
| Conjunctival hyperemia | | | | | | | | |
| Mild | 0/3 | — | 3/3 | 2/3 | 0/3 | 1/3 | 0/3 | 1/3 |
| Moderate | 0/3 | — | 0/3 | 0/3 | 2/3 | 0/3 | 0/3 | 1/3 |
| Conjunctival discharge | | | | | | | | |
| Mild | 0/3 | — | 0/3 | 0/3 | 0/3 | 0/3 | 0/3 | 1/3 |
| Preretinal (vitreal) hemorrhage | 0/3 | 3/3 | 0/3 | 0/3 | 0/3 | 0/3 | 0/3 | 0/3 |
| Retinal fold | 0/3 | — | 1/3 | 1/3 | 1/3 | 0/3 | 0/3 | 0/3 |
| Pigmentation at injection site | 0/3 | — | 2/3 | 3/3 | 3/3 | 3/3 | 3/3 | 3/3 |
| Tapetal alteration outside of the treated area | 0/3 | — | 1/3 | 1/3 | 1/3 | 1/3 | 1/3 | 1/3 |

—, not applicable.

Supplementary Table S2. Ophthalmic examination findings of eyes injected with AGTC-501 at 1.2×10^{11} vg/mL (low dose)

| <i>Finding</i> | <i>Predose</i> | <i>Study Day 0</i> | <i>Study Week 1</i> | <i>Study Week 2</i> | <i>Study Week 6</i> | <i>Study Week 10</i> | <i>Study Week 15</i> | <i>Study Week 20</i> |
|--|----------------|--------------------|---------------------|---------------------|---------------------|----------------------|----------------------|----------------------|
| Conjunctival hyperemia | | | | | | | | |
| Mild | 0/4 | — | 2/4 | 1/4 | 3/4 | 2/4 | 1/4 | 2/4 |
| Moderate | 0/4 | — | 1/4 | 1/4 | 0/4 | 1/4 | 0/4 | 0/4 |
| Conjunctival chemosis | | | | | | | | |
| Mild | 0/4 | — | 0/4 | 0/4 | 0/4 | 1/4 | 0/4 | 1/4 |
| Pigmentation at injection site | 0/4 | — | 2/4 | 3/4 | 4/4 | 4/4 | 4/4 | 3/4 |
| Tapetal alteration outside of the treated area | 0/4 | — | 1/4 | 1/4 | 2/4 | 2/4 | 2/4 | 2/4 |
| Retinal atrophy | 0/4 | — | 0/4 | 0/4 | 0/4 | 1/4 ^A | 0/4 | 1/4 ^{a,b} |
| Bleb border visible | 0/4 | — | 0/4 | 0/4 | 0/4 | 1/4 | 2/4 | 3/4 |

^ADetected by OCT.

^bAt week 19.

—, no observations noted; AGTC, Applied Genetic Technologies Corporation; OCT, optical coherence tomography; vg, vector genome.

Supplementary Table S3. Ophthalmic examination findings of eyes injected with AGTC-501 at 6.0×10^{11} vg/mL (mid dose)

| <i>Finding</i> | <i>Predose</i> | <i>Study Day 0</i> | <i>Study Week 1</i> | <i>Study Week 2</i> | <i>Study Week 6</i> | <i>Study Week 10</i> | <i>Study Week 15</i> | <i>Study Week 20</i> |
|--|----------------|--------------------|---------------------|---------------------|---------------------|----------------------|----------------------|----------------------|
| Conjunctival hyperemia | | | | | | | | |
| Mild | 0/4 | — | 1/4 | 0/4 | 2/4 | 2/4 | 1/4 | 0/4 |
| Moderate | 0/4 | — | 0/4 | 0/4 | 0/4 | 0/4 | 0/4 | 1/4 |
| Conjunctival chemosis | | | | | | | | |
| Mild | 0/4 | — | 1/4 | 1/4 | 0/4 | 1/4 | 0/4 | 0/4 |
| Incipient posterior Y suture cataract | 0/4 | — | 0/4 | 0/4 | 0/4 | 1/4 | 1/4 | 1/4 |
| Vitreal blood clot | 0/4 | — | 1/4 | 0/4 | 0/4 | 0/4 | 0/4 | 0/4 |
| Preretinal (vitreal) hemorrhage | 0/4 | 1/4 | 0/4 | 0/4 | 0/4 | 0/4 | 0/4 | 0/4 |
| Pigmentation at injection site | 0/4 | — | 3/4 | 3/4 | 4/4 | 4/4 | 4/4 | 4/4 |
| Tapetal alteration outside of the treated area | 0/4 | — | 3/4 | 3/4 | 2/4 | 2/4 | 2/4 | 2/4 |
| Bleb border visible | 0/4 | — | 0/4 | 0/4 | 1/4 | 2/4 | 3/4 | 3/4 |
| Retinal detachment | 0/4 | — | 0/4 | 0/4 | 0/4 | 0/4 | 0/4 | 1/4 ^{a,b} |

^ADetected by OCT.

^bAt week 19.

—, no observations noted.