

Supplementary Table S4. Ophthalmic examination findings of eyes injected with AGTC-501 at 3.0 × 10¹² vg/mL (high dose)

Finding	Pre-dose	Study Day 0	Study Week 1	Study Week 2	Study Week 6	Study Week 10	Study Week 15	Study Week 20
Conjunctival hyperemia								
Mild	0/5	—	3/5	2/5	2/5	1/5	2/5	2/5
Conjunctival chemosis								
Mild	0/5	—	0/5	0/5	0/5	1/5	0/5	0/5
Corneal epithelial deposit	0/5	—	0/5	0/5	0/5	2/5	2/5	1/5
Vitreous strand/fibrin	0/5	—	0/5	0/5	0/5	1/5	1/5	1/5
Vitreous cells	0/5	—	0/5	0/5	0/5	2/5	2/5	1/5
Vitreous blood clot	0/5	—	1/5	1/5	1/5	1/5	0/5	0/5
Retinal fold	0/5	—	1/5	1/5	2/5	1/5	1/5	1/5
Changes in transparency of the vitreous	0/5	—	0/5	0/5	0/5	1/5	0/5	0/5
Pigmentation at injection site	0/5	—	4/5	4/5	4/5	5/5	5/5	5/5
Bleb border visible	0/5	—	0/5	0/5	1/5	4/5	4/5	4/5
Retinal detachment	0/5	—	0/5	0/5	0/5	2/5 ^A	0/5	1/5 ^{a,b}
Chorioretinitis	0/5	—	0/5	0/5	0/5	1/5	1/5	1/5
Retinal atrophy	0/5	—	0/5	0/5	0/5	0/5	0/5	1/5
Tapetal alteration outside of the treated area	0/5	—	3/5	3/5	3/5	3/5	3/5	3/5
Dilated retinal vessels	0/5	—	0/5	0/5	0/5	2/5	1/5	1/5

^ADetected by OCT.

^bAt week 19.

—, no observations noted.

Supplementary Table S5. Gross pathology findings summary

Finding	Vehicle	Low Dose	Mid Dose	High Dose
Skin alopecia	1/3	1/4	1/4	0/5
Umbilical hernia	0/3	0/4	1/4	0/5
Blepharitis	0/3	0/4	1/4	0/5
Submandibular lymph nodes enlargement	0/3	0/4	1/4	0/5

Supplementary Table S6. Histopathology findings summary

Finding	Vehicle	Low Dose	Mid Dose	High Dose
Skin				
Demodicosis	1/3	1/4	1/4	0/5
Juvenile sterile granulomatous dermatitis and lymphadenitis	0/3	0/4	1/4	0/5
Lungs				
Multifocal mononuclear infiltrates (left and right)	1/3	0/4	1/4	0/5
Minimal focal mononuclear infiltrate (right)	1/3	0/4	0/4	0/5
Minimal mononuclear infiltrates (left and right)	0/3	0/4	1/4	2/5
Moderate mononuclear infiltrates (left and right)	0/3	1/4	0/4	0/5
Minimal multifocal mononuclear infiltrates (left and right)	0/3	1/4	0/4	0/5
Liver				
Focal microabscess	1/3	0/4	0/4	0/5
Focal microgranuloma	0/3	0/4	0/4	1/5
Kidneys				
Minimal focal mononuclear infiltrate (left)	1/3	0/4	0/4	0/5
Minimal mononuclear infiltrates (left and right)	0/3	1/4	0/4	0/5
Optic tract				
Focal mononuclear infiltrate	0/3	0/4	0/4	1/5

Supplementary Table S7. Ocular histopathology findings summary

Finding	Vehicle		Low Dose		Mid Dose		High Dose	
	Tx	UnTx	Tx	UnTx	Tx	UnTx	Tx	UnTx
Few perivascular conjunctival and episcleral aggregates of lymphocytes and plasma cells	0/3	0/3	1/4	1/4	0/4	0/4	0/5	0/5
Minimal perivascular lymphoplasmacytic infiltrates within the retinal ganglion cell layer.	0/3	0/3	0/4	0/4	0/4	0/4	0/5	1/5
Perivascular infiltrate of small lymphocytes and plasma cells within the retinal ganglion cell layer and the optic nerve head.								
Minimal focal	0/3	0/3	0/4	0/4	0/4	0/4	1/5	0/5
Mild	0/3	0/3	0/4	0/4	0/4	0/4	1/5	0/5
Multifocal moderate + milder similar infiltrate in the inner nuclear layer and choroid	0/3	0/3	0/4	0/4	0/4	0/4	1/5	0/5
Moderate	0/3	0/3	0/4	0/4	0/4	0/4	2/5	0/5
Multifocal outer nuclear layer to complete retinal atrophy	0/3	0/3	0/4	0/4	0/4	0/4	1/5	0/5

Tx, treated; UnTx, untreated.