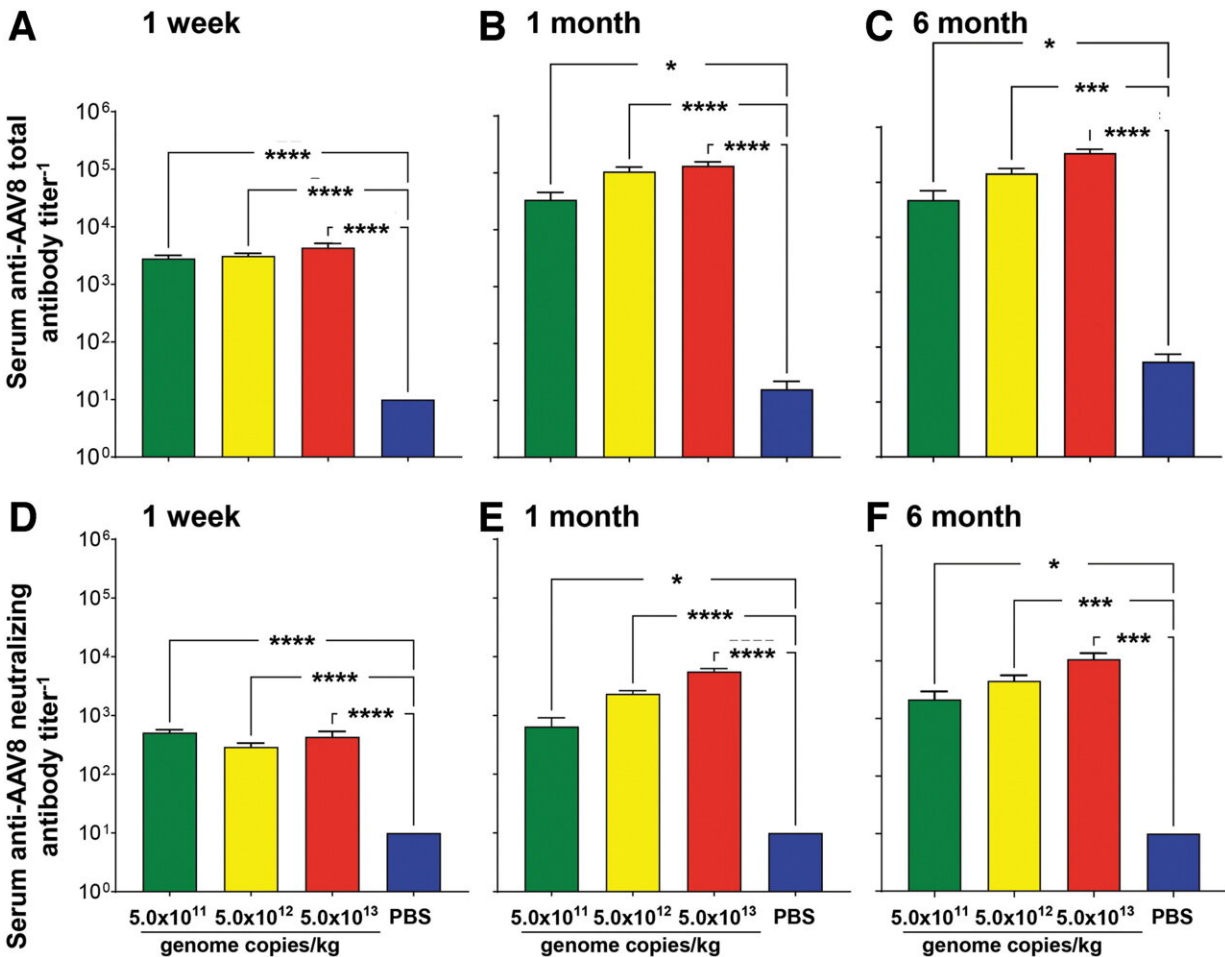


Second generation gene therapy for alpha 1-antitrypsin deficiency

February 22 2023



Serum anti-AAV8 total and neutralizing antibody titers in mice after intravenous administration of AAV8hAAT(AVL). Assessment of total anti-AAV8 and neutralizing anti-AAV8 antibody titers in treated mice for humoral response evoked by IV administration of AAV8hAAT(AVL). The total antibody titer is expressed as the reciprocal of the serum dilution, while the neutralizing antibody

titer is expressed as the reciprocal of serum dilution at which 50% inhibition of AAV8Luc was observed. (A–C) AAV8 total and (D–F) neutralizing antibody titers at 1 week, 1 month, and 6 months after single IV administration of PBS or AAV8hAAT(AVL), color coded by treatment (*blue*, PBS; *green*, AAV8hAAT(AVL), 5.0×10^{11} gc/kg dose; *yellow*, AAV8hAAT(AVL), 5.0×10^{12} gc/kg dose; *red*, AAV8hAAT(AVL), 5.0×10^{13} gc/kg dose). Titers are expressed as the geometric mean \pm SEM for 5F/5M for each time point (gender data combined). (A, D) One-week cohort. (B, E) One-month cohort. (C, F) Six-month cohort. All mice were treated at 8 weeks of age \pm 3 days. Comparisons between vector doses and age-matched PBS controls were assessed by unpaired *t*-test for effect of treatment dosage versus control. **p*

Citation: Second generation gene therapy for alpha 1-antitrypsin deficiency (2023, February 22) retrieved 4 February 2024 from <https://medicalxpress.com/news/2023-02-generation-gene-therapy-alpha-antitrypsin.html>

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