OMTM, Volume *17*

Supplemental Information

Timing of Intensive Immunosuppression Impacts Risk of Transgene Antibodies after AAV Gene Therapy in Nonhuman Primates

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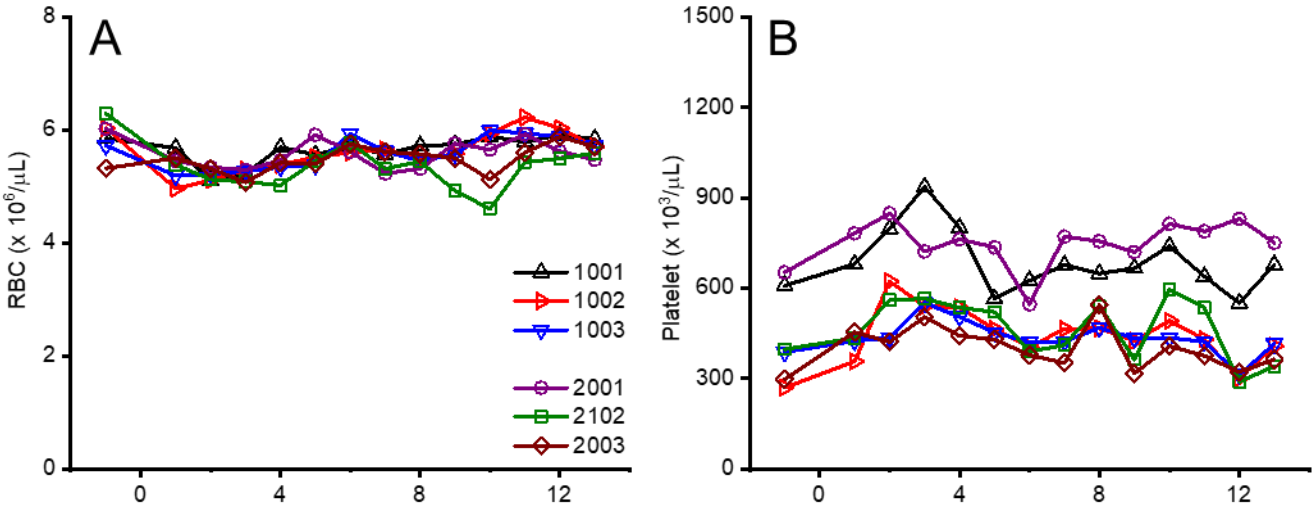
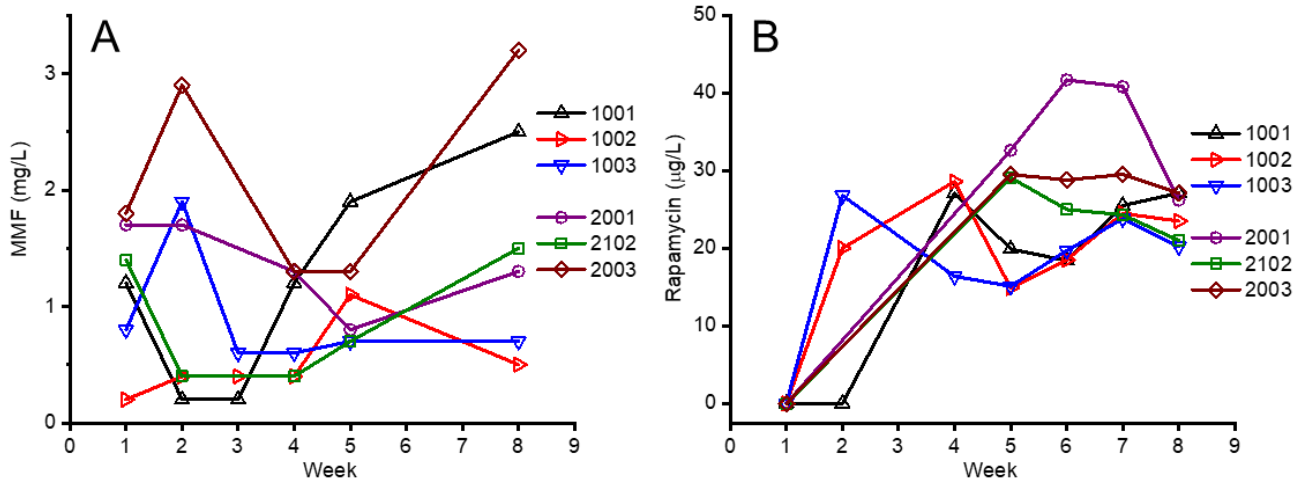
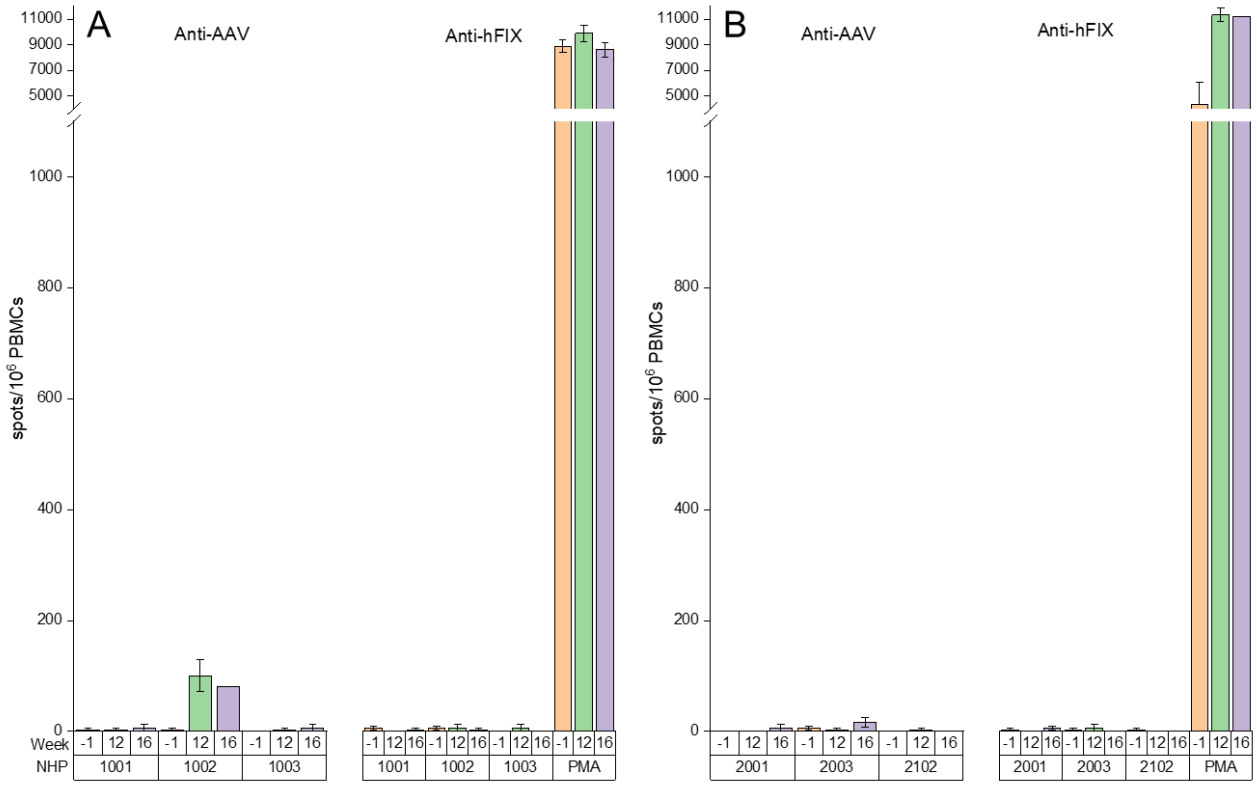


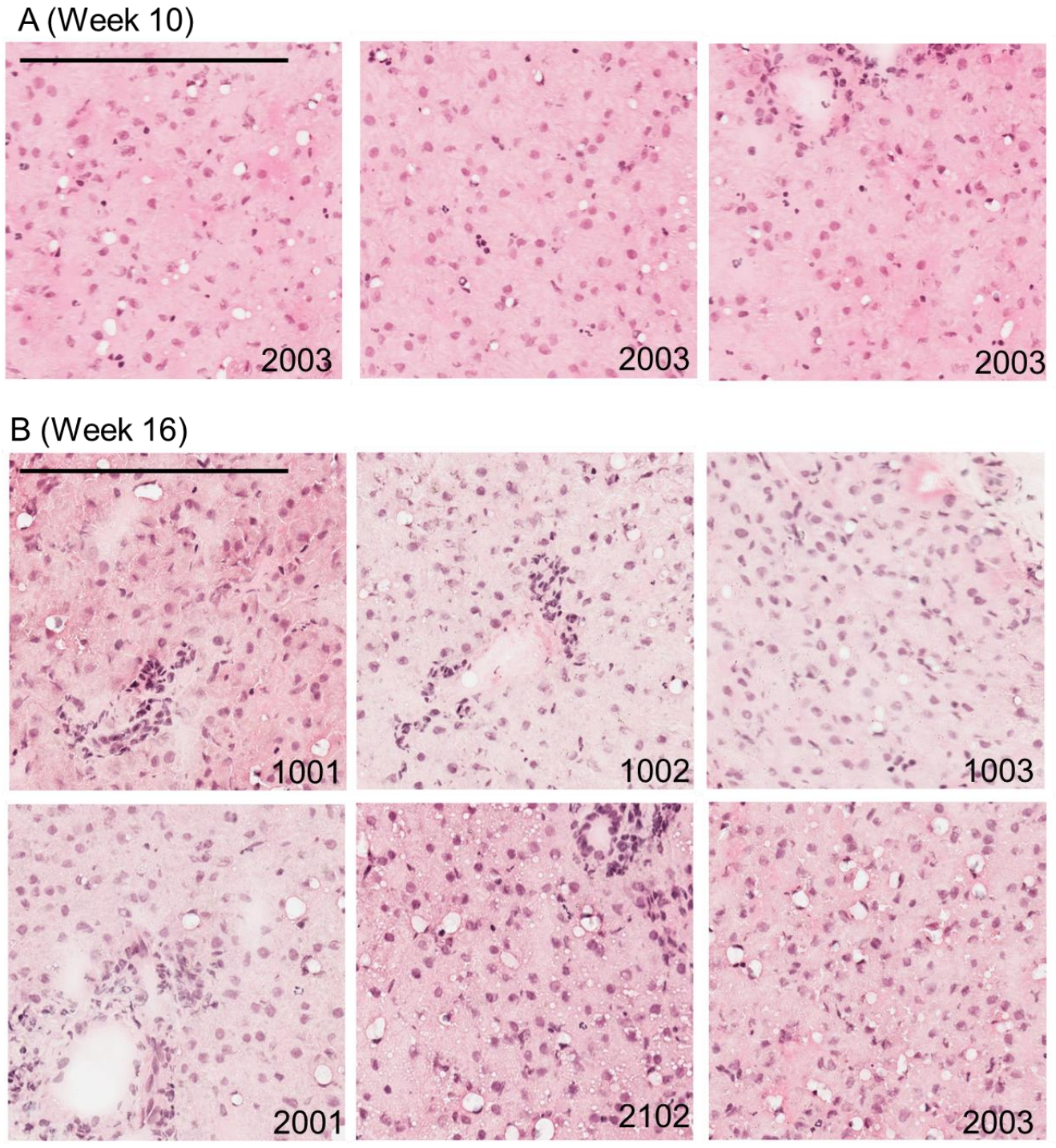
Supplementary Figure 1: Hematological parameters of NHPs before and after vector administration. The red blood cell (RBC) count (A) and platelet count (B) of each NHP as indicated.



Supplementary Figure 2: Plasma drug levels. Plasma levels of MMF (A) and rapamycin (B) for each NHP through IS withdraw at week 8.



Supplementary Figure 3: Cellular immune response to AAV and hFIX by ELISpot analysis before and after vector administration. ELISpot analysis of PBMCs from NHPs in Group 1 (A), early ATG, or Group 2 (B), delayed ATG, collected at week -1, 12, and 16 and stimulated with either hFIX protein or empty AAV2 capsid particles, and detected based on IFN-γ secretion. Phorbol myristate (PMA) is a positive control. Bars represent mean of triplicate measurement and error bars are ± SEM. At some time points, no spots were detected.



Supplementary Figure 4: Histology of liver biopsies. Representative hematoxylin and eosin staining of liver biopsies obtained at 10 weeks (A) or 16 weeks (B) after vector administration of each NHP as indicated. Black bar indicates 200 µm.