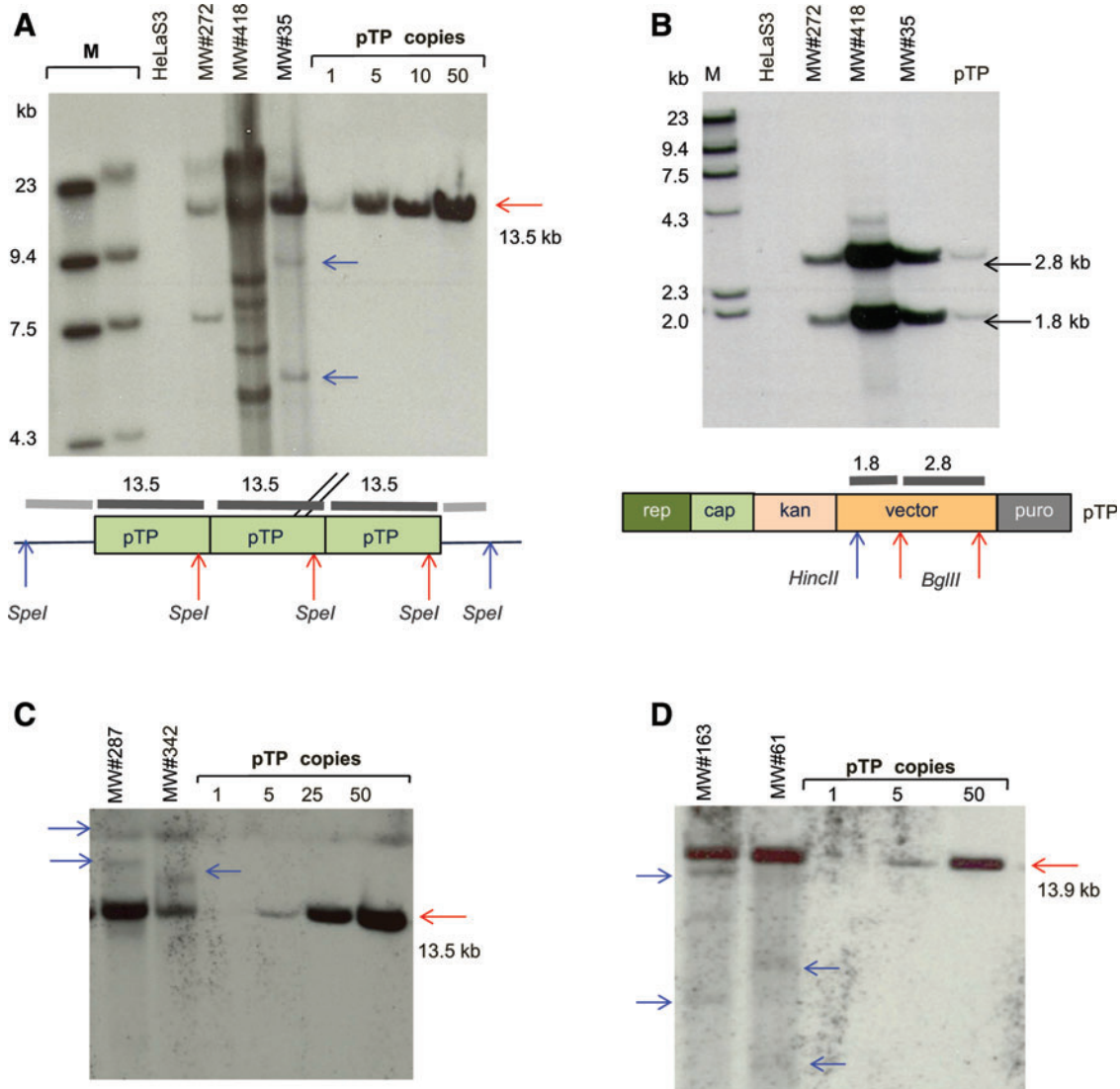


Supplementary Data



Supplementary Figure S1. Characterization of genomic DNA isolated from selected MMs by Southern blot analysis. **(A)** Analysis of genomic DNA from AAVrh8R/FVIII-5.1 MMs. Genomic DNA from MW#272, #418, and #35 was isolated and digested with *SpeI*, which cuts once in the vector-*rep-cap*-containing pTP plasmid and generates a fragment with a 13.5 kb-unit length (diagram below). The copy number standard was generated by digesting the parental pTP plasmid with *SpeI*. Flanking fragments are shown (blue arrows). **(B)** Integrity of the vector genome in the AAVrh8R/FVIII-5.1 MMs. The presence of the full-length expression cassette was assessed by digestion with the restriction enzymes *HincII* and *BglII*, which cut at the 5', middle, and 3' regions of the expression cassette to generate 1.8 and 2.8 kb fragments (diagram below).²² **(C)** Analysis of the genomic DNA from AAV8/FVIII-5.1 MMs. **(D)** Analysis of the genomic DNA from AAVrh8R/FVIII-5.4 MMs. *SpeI* digestion of the parental pTP plasmid generates a fragment with a 13.9 kb unit length.