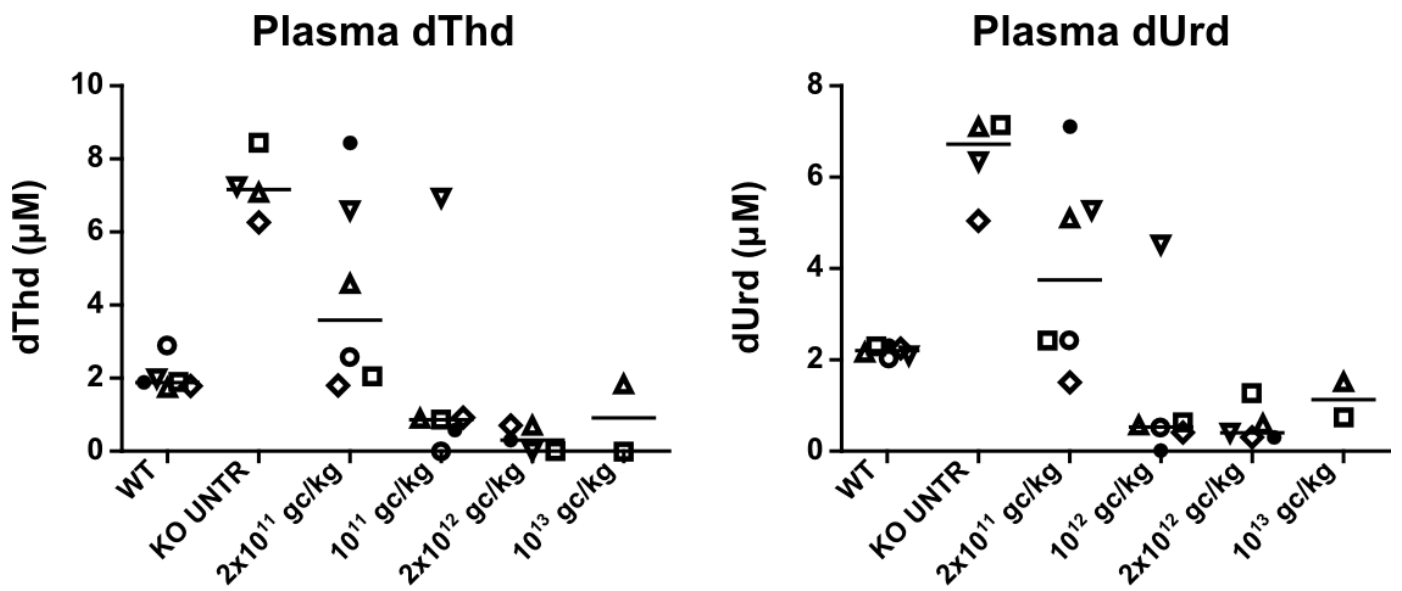
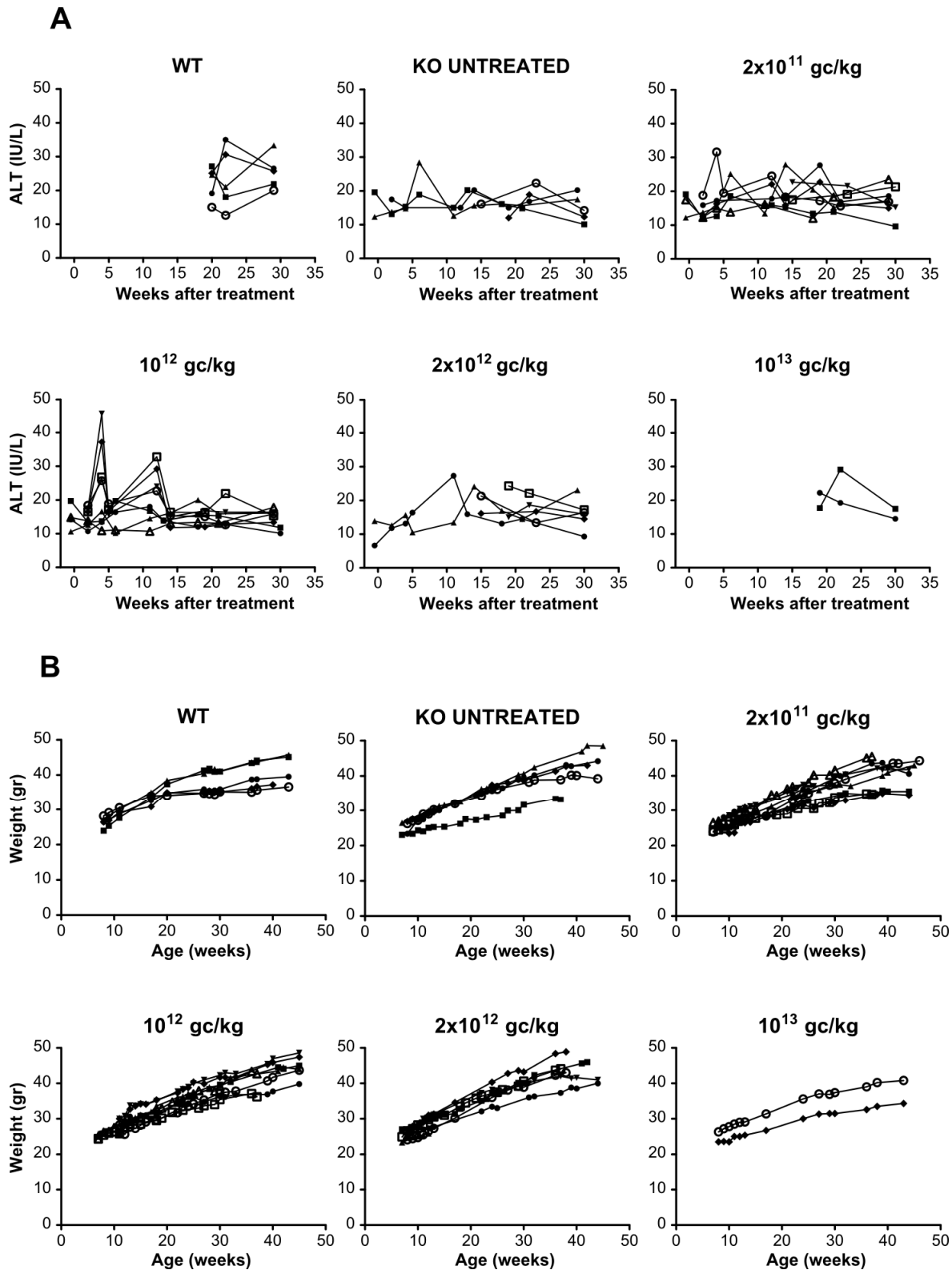


Supplementary Figure S1. Schematic representation of AAV2/8-TBG-hcTYMP. BGH pA, bovine growth hormone polyadenylation signal; hcTYMP, human thymidine phosphorylase coding sequence; ITR, inverted terminal repeats; TBG, thyroxine-binding globulin promoter; WPRE, woodchuck hepatitis virus posttranscriptional regulatory element.



Supplementary Figure S2. Plasma nucleoside levels at the completion of the study. Plasma dThd and dUrd concentration were measured in samples from intracardiac blood 34 weeks after treatment. Horizontal lines represent median values.



Supplementary Figure S3. Absence of liver toxicity in AAV treated mice. (A) Plasma alanine aminotransferase activity (ALT) measured before treatment and at different time-points after AAV injection. (B) Body weight of wt, and treated and non-treated KO mice