



Supplementary information, Figure S5. CRISPR/Cas9 system rescues the manifestations of PRKAG2 cardiac syndrome in transgenic mouse model.

(A) Heart weight. One-way ANOVA was used to analyze overall differences between all groups (n=11, 16 and 10 for control, AAV9-EGFP or AAV9-Cas9/sgRNA-1 treated Tg-H530R mice respectively). Unpaired two-tailed Student's t-test for single comparison, ***p<0.001.

(B) H&E staining and PAS staining of paraffin embedded sections.

(C) Glycogen measurement. One-way ANOVA was used to analyze overall differences between all groups (n=10, 12 and 11 for control, AAV9-EGFP or AAV9-Cas9/sgRNA-1 treated Tg-H530R mice respectively). Unpaired two-tailed Student's t-test for single comparison, ***p<0.001.

(D) Transmission electron microscopy of ultrathin sections of hearts from 12 week-old mice as indicated.