

## Supplementary information, Figure S4. AAV9-delivered CRISPR/Cas9 system to neonatal transgenic hearts achieves mutagenesis at target mutation site.

(A) Diagrams showing the AAV vectors expressing Cas9 and sgRNAs respectively.

(B) Schematic of candidate sgRNA target sites in exogenous mutant PRKAG2 transgene.

(C) Screening of candidate sgRNA by Surveyor assay. Arrow denotes the on-target cleavage products.

(D) Tissue expression analysis of Cas9 in mice 2 months after injection with AAV9-Cas9/sgRNA-1.

(E) Sequence of PCR product amplified from the sgRNA-1 on-target site. Underline denotes the sgRNA

sequence, and arrow denotes the Cas9 cleavage site.

(F) Resulting DNA sequences of on-target sites with indels.

(G) Mutagenesis analyzed by Surveyor assay. Arrow denotes the on-target cleavage products.

(H) Off-target analysis by Surveyor assay. The information about OT-1 to OT-10 is listed in Supplementary information Table S3.