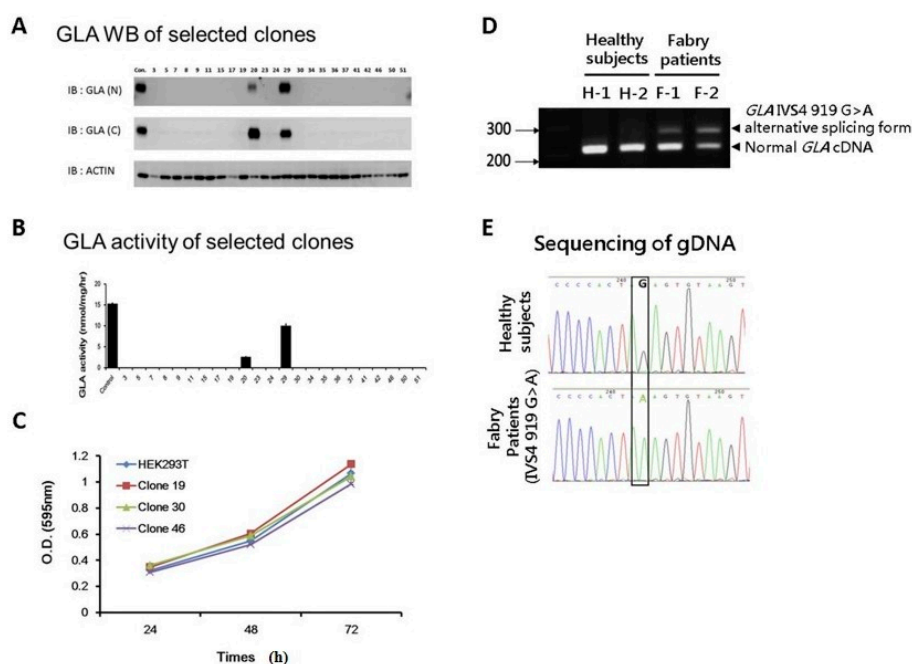
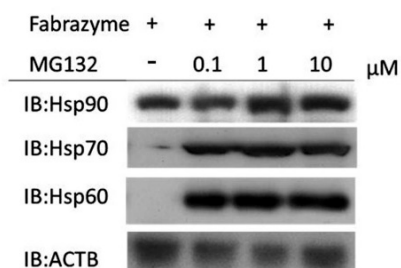


# Supplementary Materials: Using CRISPR/Cas9-Mediated *GLA* Gene Knockout as an In Vitro Drug Screening Model for Fabry Disease

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**Figure S1.** The GLA protein expression levels (A) and the GLA enzyme activity (B) were determined in 23-selected CRISPR/Cas9-mediated *GLA* knockout clones in HEK-293T cells; (C) Cell proliferation was determined in the parental HEK-293T cells and the GLA-null cell clone 19, 30 and 46; (D) The genotype analysis of the *GLA* cDNA at the junction between exon 4 and 5 was examined by RT-PCR amplification of 0.1 mg of total RNA that prepared from the FD patient-derived fibroblasts and from the healthy subject-derived fibroblasts. The primers 5'-GTCCTTGGCCCTGAATAG-3' and 5'-GTCCAGCAACATCAACAATT-3' were used for PCR, and then the results were analyzed by agarose gel electrophoresis; (E) Sequence analysis confirmed the nucleotide of *GLA* IVS4 + 919 is "G" (highlight in the black square) in the fibroblasts derived from healthy subjects comparing to "A" in the fibroblasts derived from patients with FD > A mutation in the FD patient-derived fibroblasts (represented as IVS4 919 G > A mutant).



**Figure S2.** Protein expression levels of Hsp90, Hsp70, and Hsp60 were determined by immunoblot analysis in the GLA-null cells co-treating 0.1 μM Fabrazyme with MG132 (0.1, 1, and 10 μM) for 24 h. β-actin (ACTB) was used as loading control in the immunoblot assay. Representative immunoblot data are presented. "+" and "-" represented the cell treatment with and without Fabrazyme/MG132 administration, respectively.