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Correction of IVS I-110(G>A)  $\beta$ -thalassemia by CRISPR/Cas- and TALEN-mediated disruption of aberrant regulatory elements in human hematopoietic stem and progenitor cells

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Contributions: P. Patsali performed molecular and cell culture work, designed, cloned and produced molecular tools, performed bioinformatics analyses and prepared all figures and the initial manuscript draft. G.T. assisted with nucleofections and performed deep sequencing experimentation and related scoring. M.R. assisted with T7E1 assays and prepared TALEN mRNAs. P. Papasavva. performed cell culture work and scored cyto centrifugation samples. C.C.L. assisted in RNP preparation. C.S. assisted with lentiviral constructs. S.C. and M.S. coordinated sampling and hematological analyses. C.M. designed molecular tools and guided study design. M.N.A. guided study design and progress. T.I.C. supervised mRNA production. C.W.L. wrote the grants, conceived and supervised the study, performed HPLC analyses and wrote the manuscript. T.C. hosted the research and guided study design. M.K. hosted the research and guided study design. P. Patsali, P. Papasavva., C.S., C.M., T.I.C., M.N.A., C.W.L., T.C. and M.K. contributed to the final version of the manuscript. All authors have approved the final version of the article. The present work has not been published previously and is not under consideration for publication elsewhere. No third-party writing or editing services were used in the creation of this manuscript.