nature medicine

SUPPLEMENTARY INFORMATION

https://doi.org/10.1038/s41591-019-0401-y

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Highly efficient therapeutic gene editing of human hematopoietic stem cells

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Supplementary Table 2. *HBB* genotype of β-thalassemia patients.

Simplified genotype	Genotype
$\beta^0\beta^0{}_{\#1}$	Homozygous codon 44 -C; αα/αα
$\beta^0\beta^0{}_{\#2}$	Homozygous IVS I-1 (G>A)
$\beta^+\beta^0{}_{\#1}$	IVS II-745 (C>G); codon 44 -C; αα/αα
$\beta^+\beta^0{}_{\#2}$	IVS I-110 (G>A); codon 39 CAG>TAG; αα/αα
$\beta^+\beta^+$	Homozygous IVS-I-5 (G>C); αα/αα
$(^A\gamma\delta\beta)^0\beta^0$	Large Chinese $(^A\gamma\delta\beta)^0$ deletion; codon 41/42 (-CTTT) ; αα/αα
$\beta^E\beta^0{}_{\#1}$	Codon 26 GAG>AAG hemoglobin E; codon 71/72 +A
$\beta^E\beta^0{}_{\#2}$	Codon 26 GAG>AAG hemoglobin E; codon 71/72 +T

Supplementary Table 3. Primary xenotransplant of Cas9 RNP edited CD34$^+$ HSPCs to 33 NBSGW recipients.

RNP	sgRNA	Donor	Cell number	Glycerol
2xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#1}$	0.4 M	2%
2xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#2}$	0.8 M	2%
2xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#3}$	0.8 M	4%
2xNLS-Cas9	*AAVS1*	$\beta^A\beta^A{}_{\#1}$	0.4 M	2%
3xNLS-Cas9	*AAVS1*	$\beta^A\beta^A{}_{\#3}$	0.4 M	2%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#1}$	0.2 M	0%
3xNLS-Cas9	1617	$\beta^S\beta^S{}_{\#1}$	0.2 M	0%
3xNLS-Cas9	1617	$\beta^S\beta^S{}_{\#2}$	0.8 M	2%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#3}$	0.8 M	2%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#3}$	0.8 M	4%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#3}$	0.8 M	6%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#4}$	0.8 M	2%
3xNLS-Cas9	1617	$\beta^A\beta^A{}_{\#5}$	0.8 M	2%