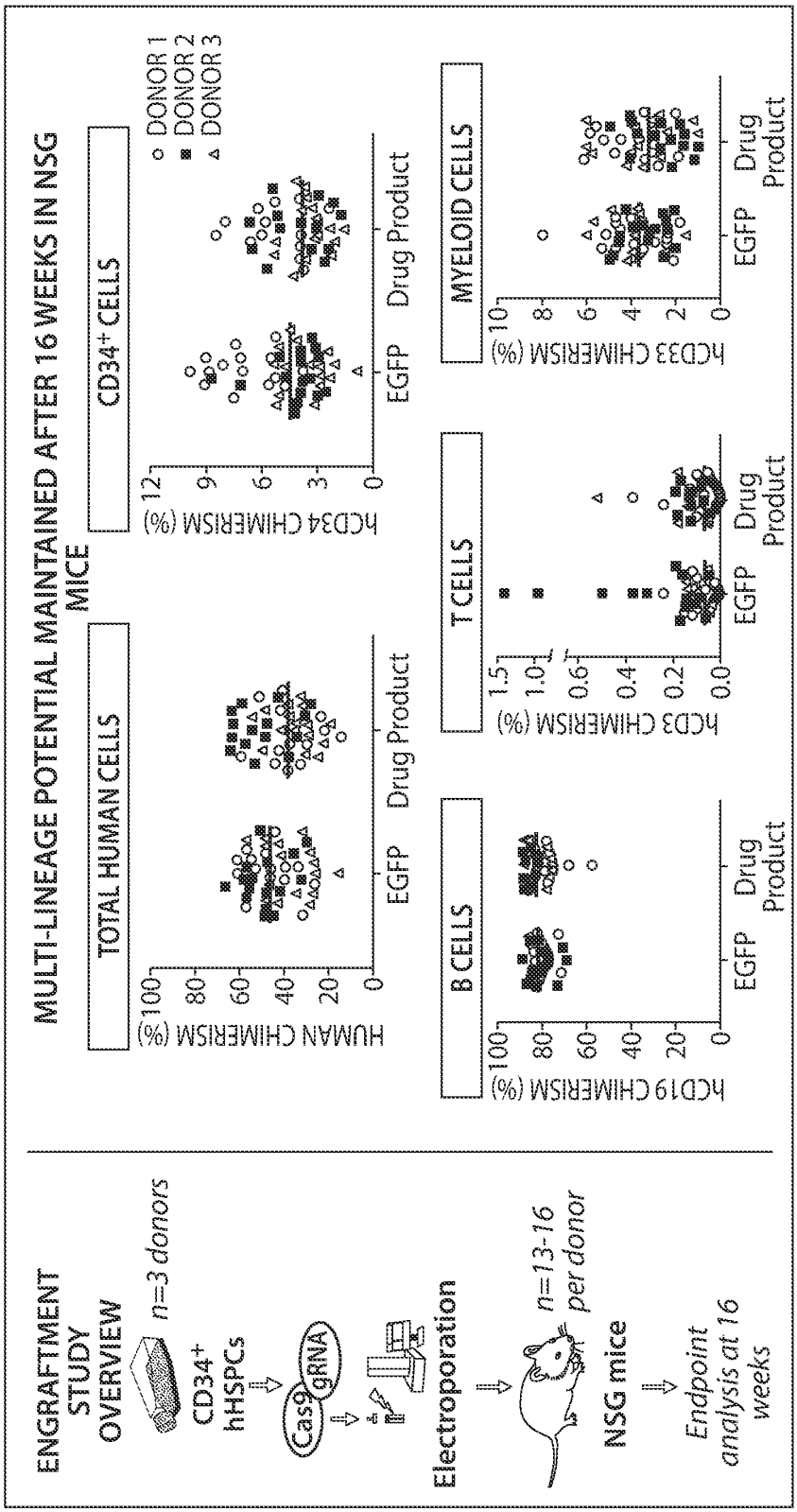

ENGRAFTMENT STUDY OVERVIEW
n=3 donors
CD34+ hHSPCs
Cas9 gRNA
Electroporation
n=13-16 per donor
NSG mice
Endpoint analysis at 16 weeks
MULTI-LINEAGE POTENTIAL MAINTAINED AFTER 16 WEEKS IN NSG MICE
TOTAL HUMAN CELLS
HUMAN CHIMERISM (%)
CD34+ CELLS
hCD34 CHIMERISM (%)
DONOR 1
DONOR 2
DONOR 3
B CELLS
hCD19 CHIMERISM (%)
T CELLS
hCD3 CHIMERISM (%)
MYELOID CELLS
hCD33 CHIMERISM (%)
EGFP
Drug Product

FIG. 1

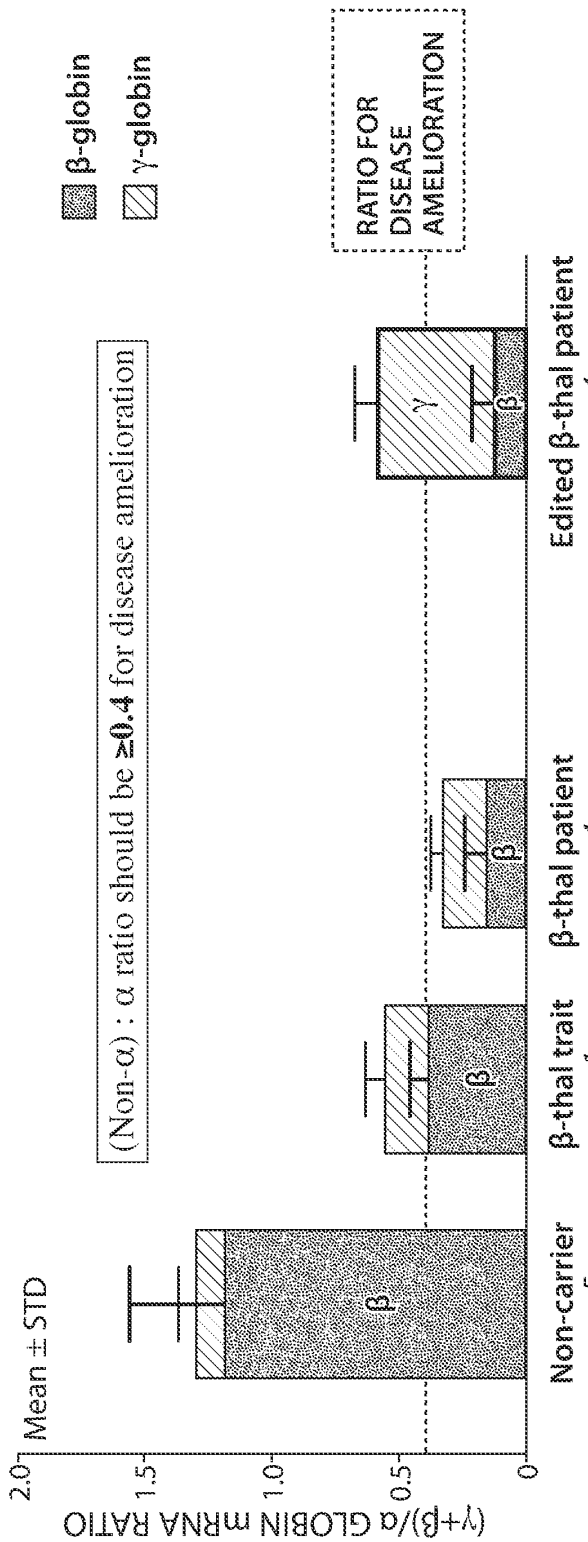

GLOBIN mRNA RATIO AFTER GENE EDITING OF PATIENT SAMPLES
β-globin
γ-globin
RATIO FOR DISEASE AMELIORATION
(Non-α) : α ratio should be ≥0.4 for disease amelioration
Mean ± STD
(γ+β)/α GLOBIN mRNA RATIO
2.0
1.5
1.0
0.5
0
β
γ
Non-carrier n=5
β-thal trait n=1
β-thal patient n=4
Edited β-thal patient n=4
ASYMPTOMATIC
SYMPTOMATIC

FIG. 2

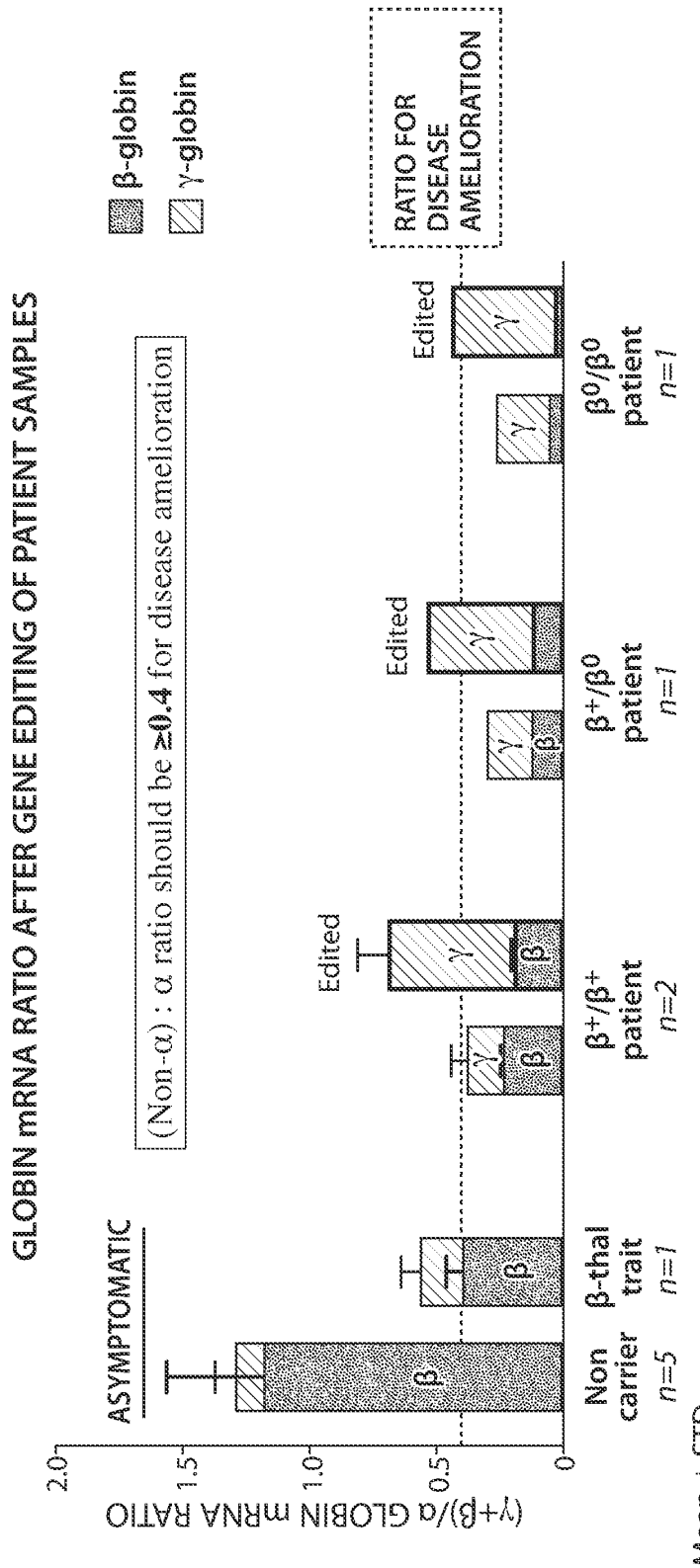

GLOBIN mRNA RATIO AFTER GENE EDITING OF PATIENT SAMPLES
β-globin
γ-globin
RATIO FOR DISEASE AMELIORATION
(Non-α) : α ratio should be ≥0.4 for disease amelioration
ASYMPTOMATIC
Edited
Edited
Edited
(γ+β)/α GLOBIN mRNA RATIO
2.0
1.5
1.0
0.5
0
Non carrier n=5
β-thal trait n=1
β+/β+ patient n=2
β+/β0 patient n=1
β0/β0 patient n=1
Mean ± STD

FIG. 3

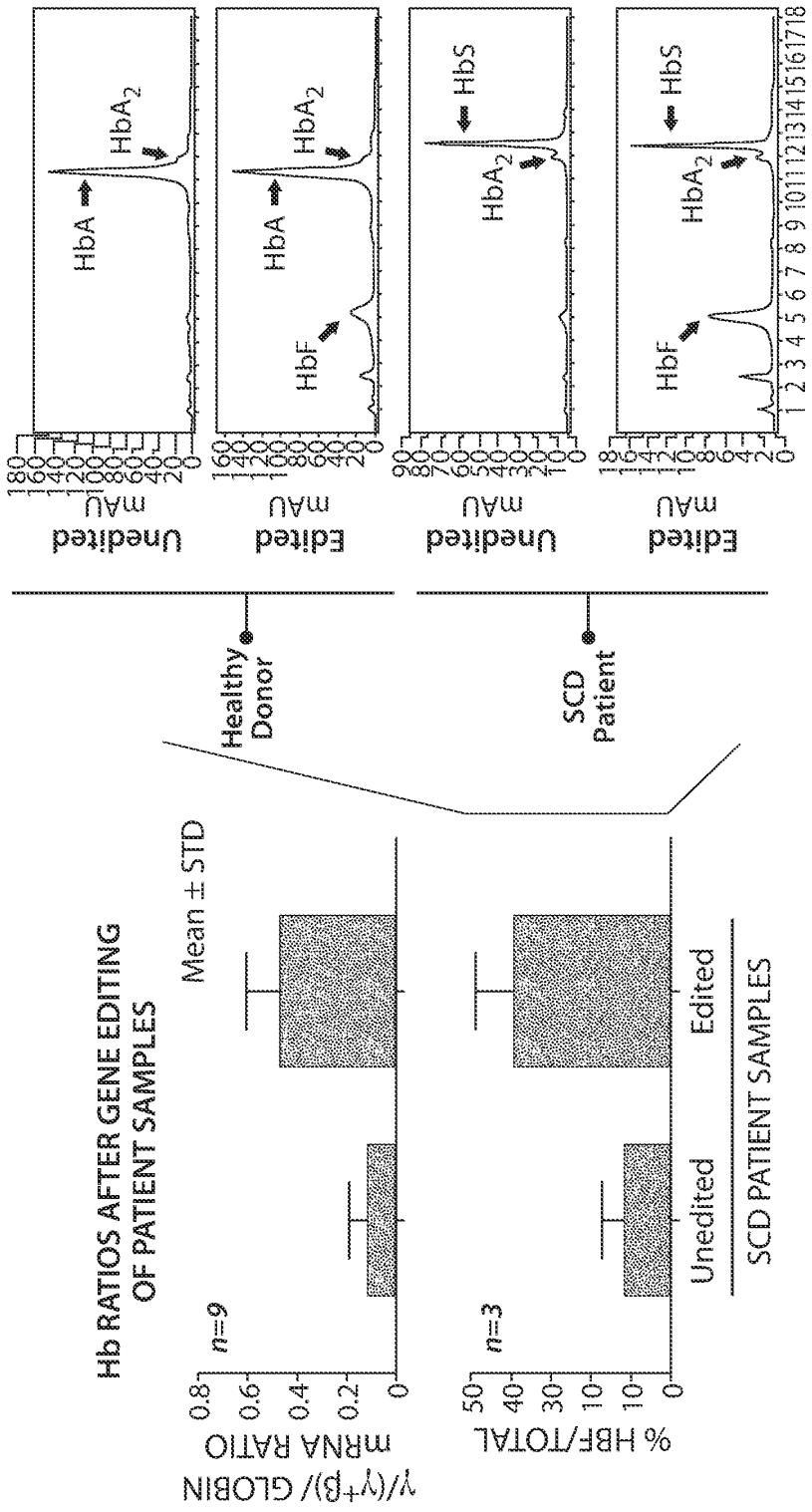

Hb RATIOS AFTER GENE EDITING
OF PATIENT SAMPLES
Mean ± STD
n=9
γ/(γ+β)/ GLOBIN
mRNA RATIO
0.8
0.6
0.4
0.2
0
n=3
% HBF/TOTAL
50
40
30
10
10
0
Unedited
Edited
SCD PATIENT SAMPLES
Healthy Donor
SCD Patient
Unedited mAU
Edited mAU
Unedited mAU
Edited mAU
HbA
HbA2
HbF
HbS
Time [min]
1 2 3 4 5 6 7 8 9 10 11 12 13 14 15 16 17 18

FIG. 4