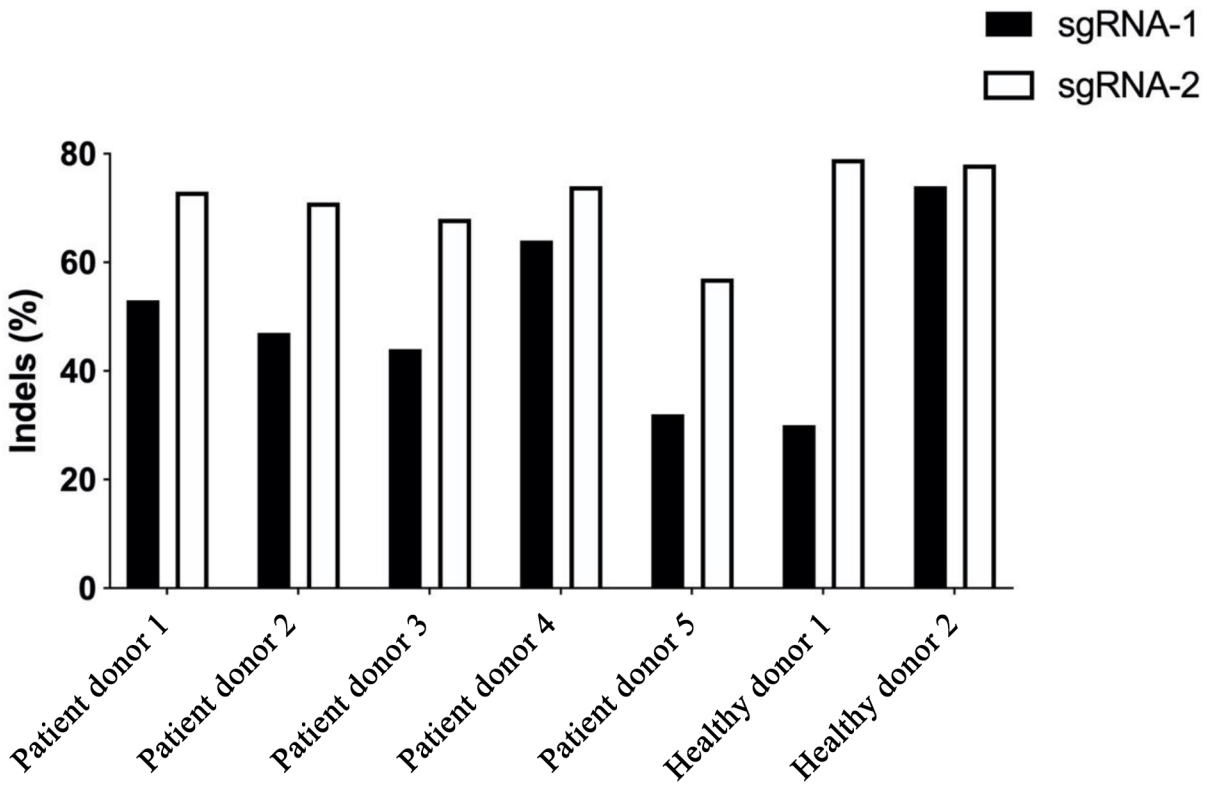

sgRNA-1
sgRNA-2
Indels (%)
80
60
40
20
0
Patient donor 1
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Healthy donor 1
Healthy donor 2

Fig. 1

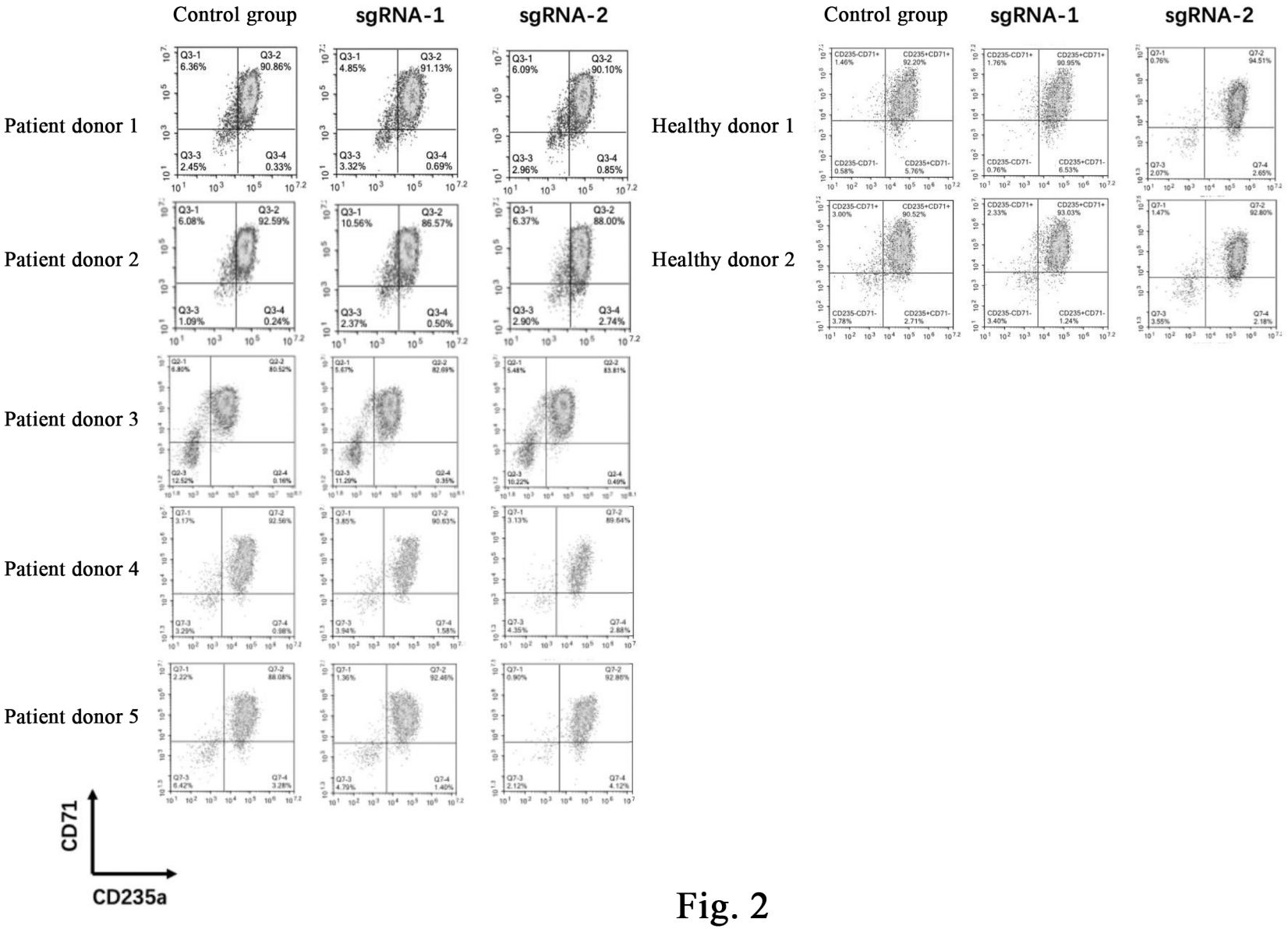

Control group
sgRNA-1
sgRNA-2
Patient donor 1
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Healthy donor 1
Healthy donor 2
CD71
CD235a

Fig. 2

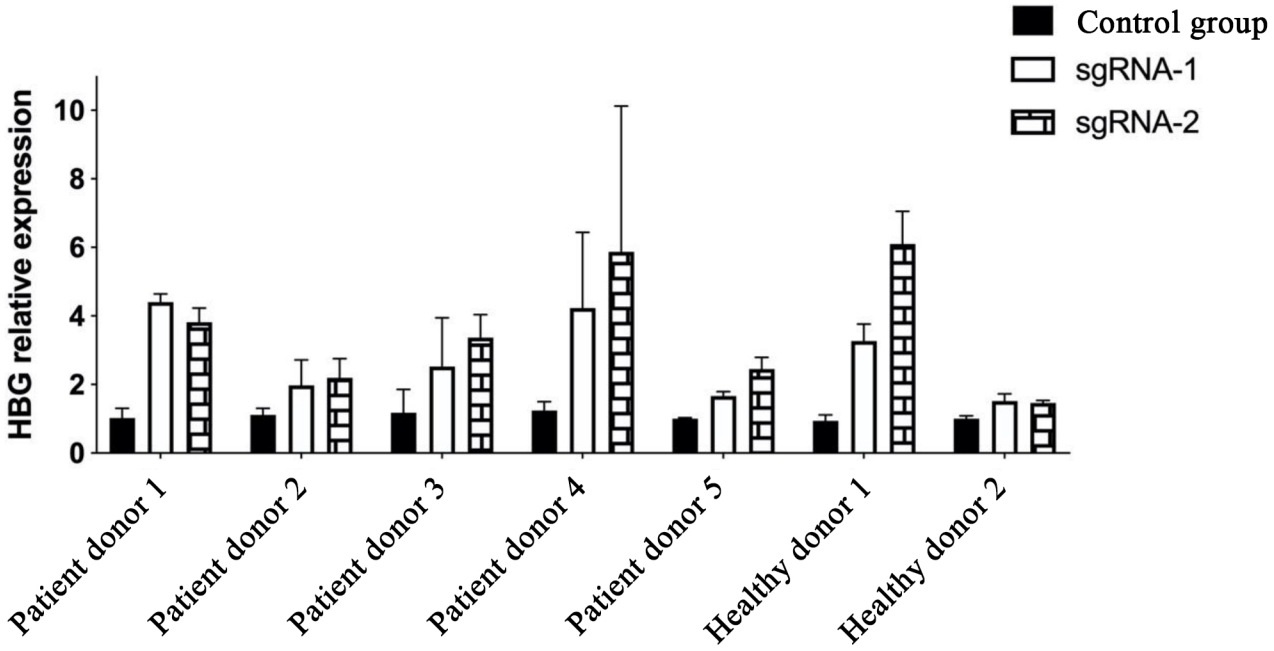

Control group
sgRNA-1
sgRNA-2
HBG relative expression
0
2
4
6
8
10
Patient donor 1
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Healthy donor 1
Healthy donor 2

Fig. 3

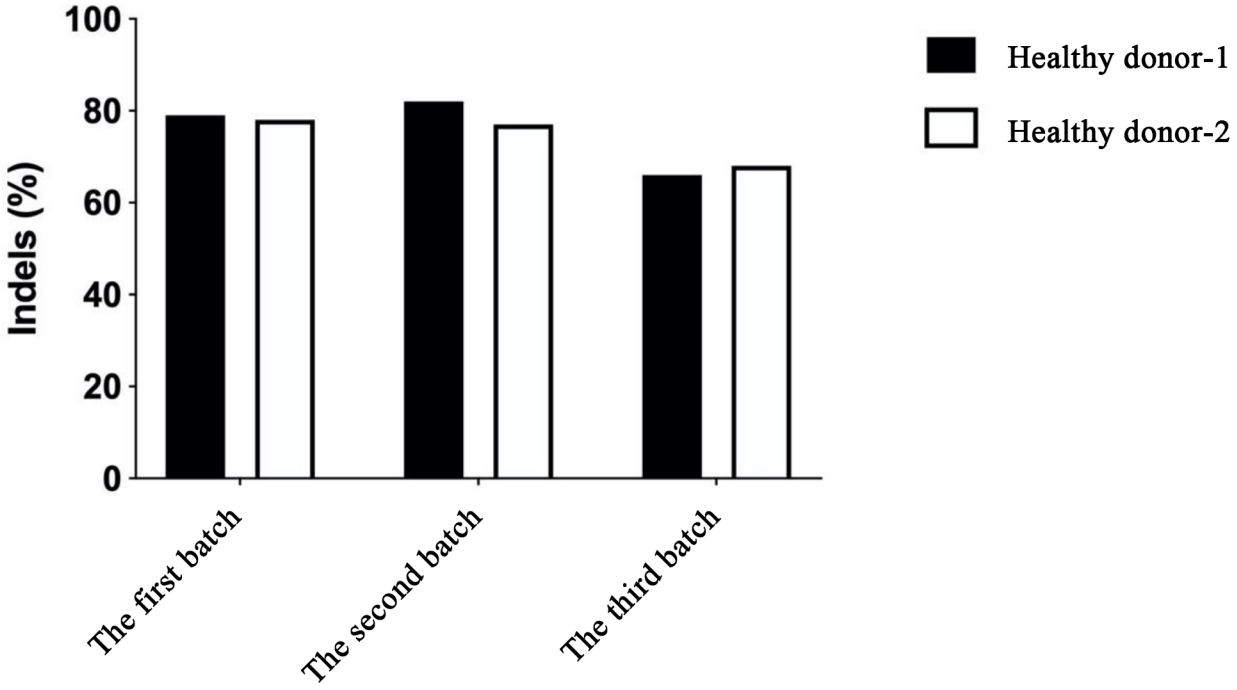

100
80
60
40
20
0
Indels (%)
Healthy donor-1
Healthy donor-2
The first batch
The second batch
The third batch

Fig. 4A

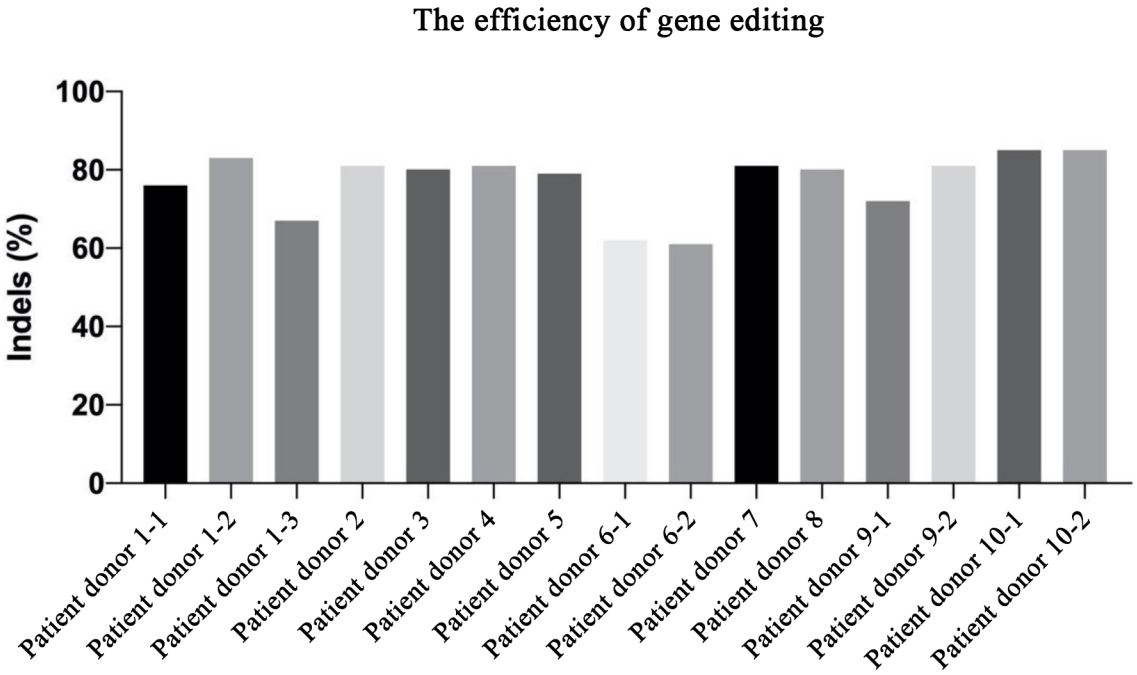

The efficiency of gene editing
100
80
60
40
20
0
Indels (%)
Patient donor 1-1
Patient donor 1-2
Patient donor 1-3
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Patient donor 6-1
Patient donor 6-2
Patient donor 7
Patient donor 8
Patient donor 9-1
Patient donor 9-2
Patient donor 10-1
Patient donor 10-2

Fig. 4B

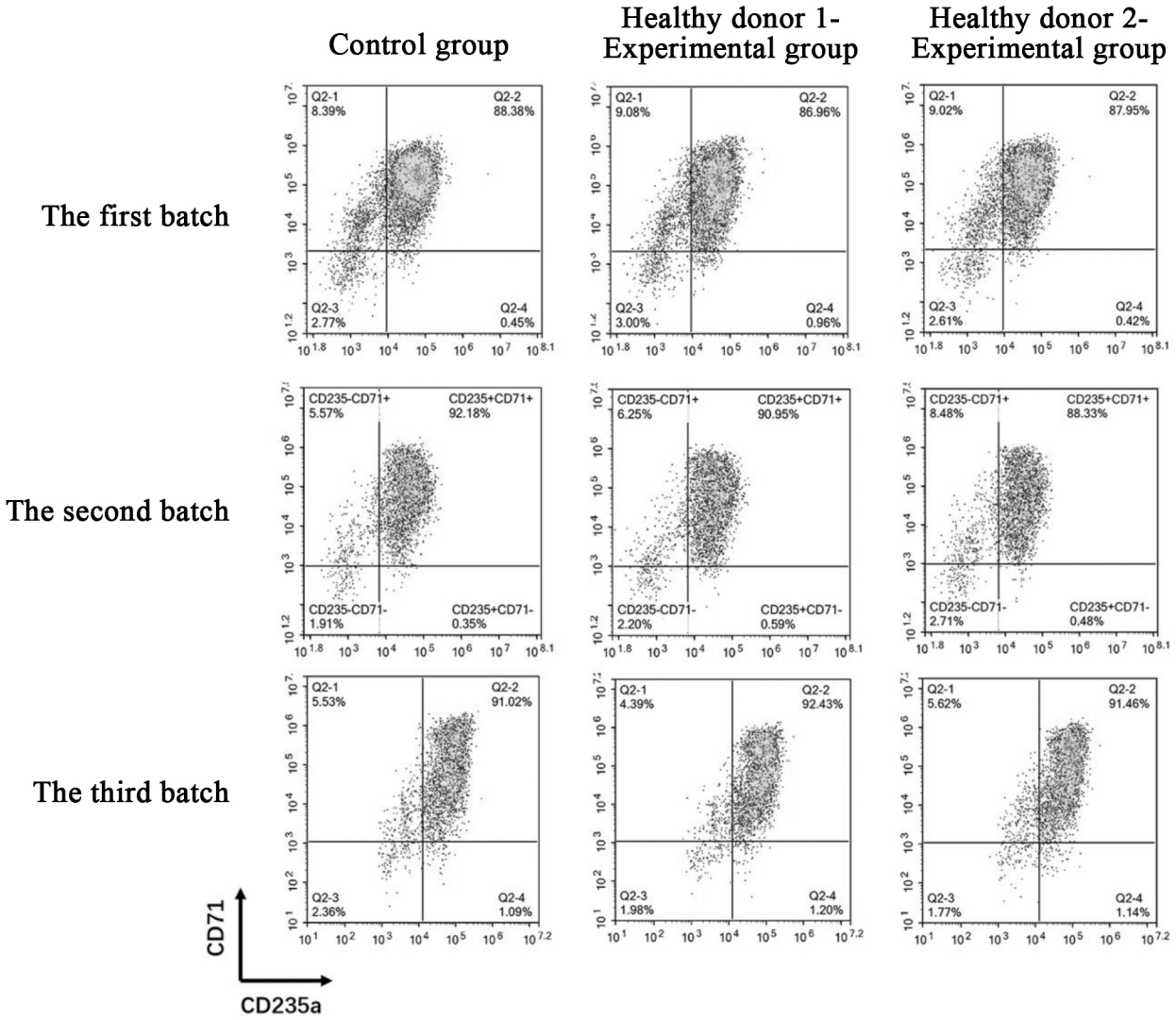

Control group
Healthy donor 1-Experimental group
Healthy donor 2-Experimental group
The first batch
The second batch
The third batch
Q2-1 8.39%
Q2-2 88.38%
Q2-3 2.77%
Q2-4 0.45%
Q2-1 9.08%
Q2-2 86.96%
Q2-3 3.00%
Q2-4 0.96%
Q2-1 9.02%
Q2-2 87.95%
Q2-3 2.61%
Q2-4 0.42%
CD235-CD71+ 5.57%
CD235+CD71+ 92.18%
CD235-CD71- 1.91%
CD235+CD71- 0.35%
CD235-CD71+ 6.25%
CD235+CD71+ 90.95%
CD235-CD71- 2.20%
CD235+CD71- 0.59%
CD235-CD71+ 8.48%
CD235+CD71+ 88.33%
CD235-CD71- 2.71%
CD235+CD71- 0.48%
Q2-1 5.53%
Q2-2 91.02%
Q2-3 2.36%
Q2-4 1.09%
Q2-1 4.39%
Q2-2 92.43%
Q2-3 1.98%
Q2-4 1.20%
Q2-1 5.62%
Q2-2 91.46%
Q2-3 1.77%
Q2-4 1.14%
CD71
CD235a

Fig. 5

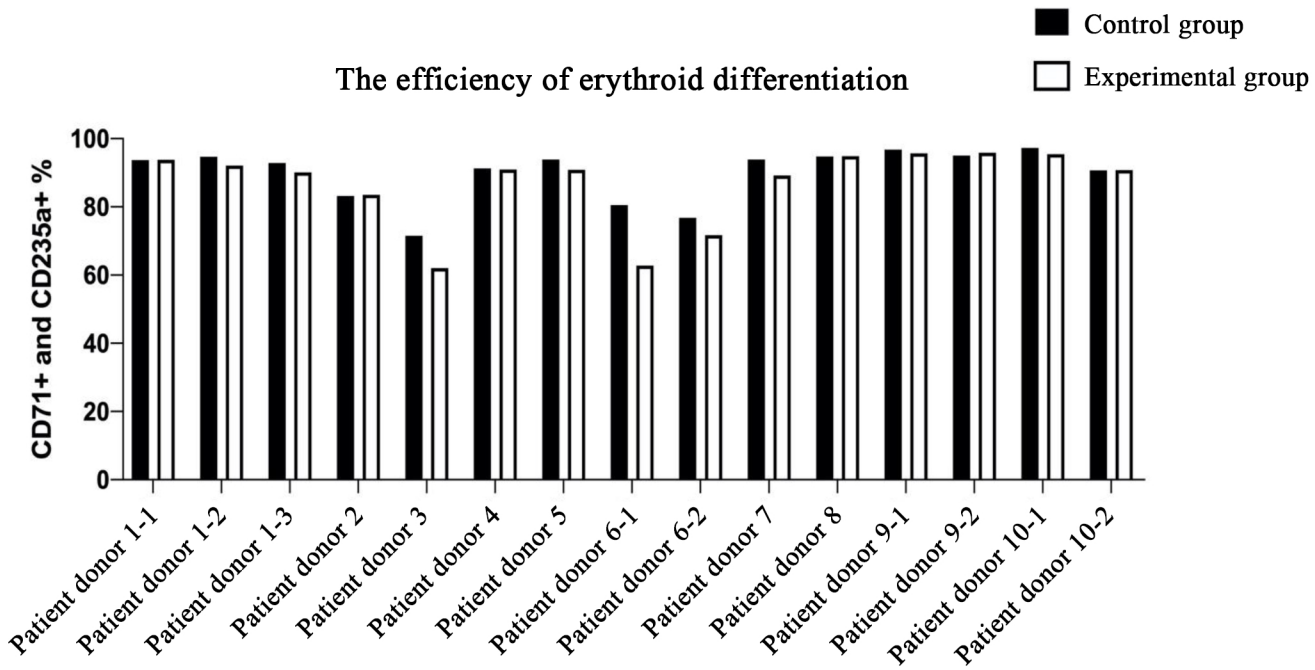

The efficiency of erythroid differentiation
Control group
Experimental group
CD71+ and CD235a+ %
100
80
60
40
20
0
Patient donor 1-1
Patient donor 1-2
Patient donor 1-3
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Patient donor 6-1
Patient donor 6-2
Patient donor 7
Patient donor 8
Patient donor 9-1
Patient donor 9-2
Patient donor 10-1
Patient donor 10-2

Fig. 6

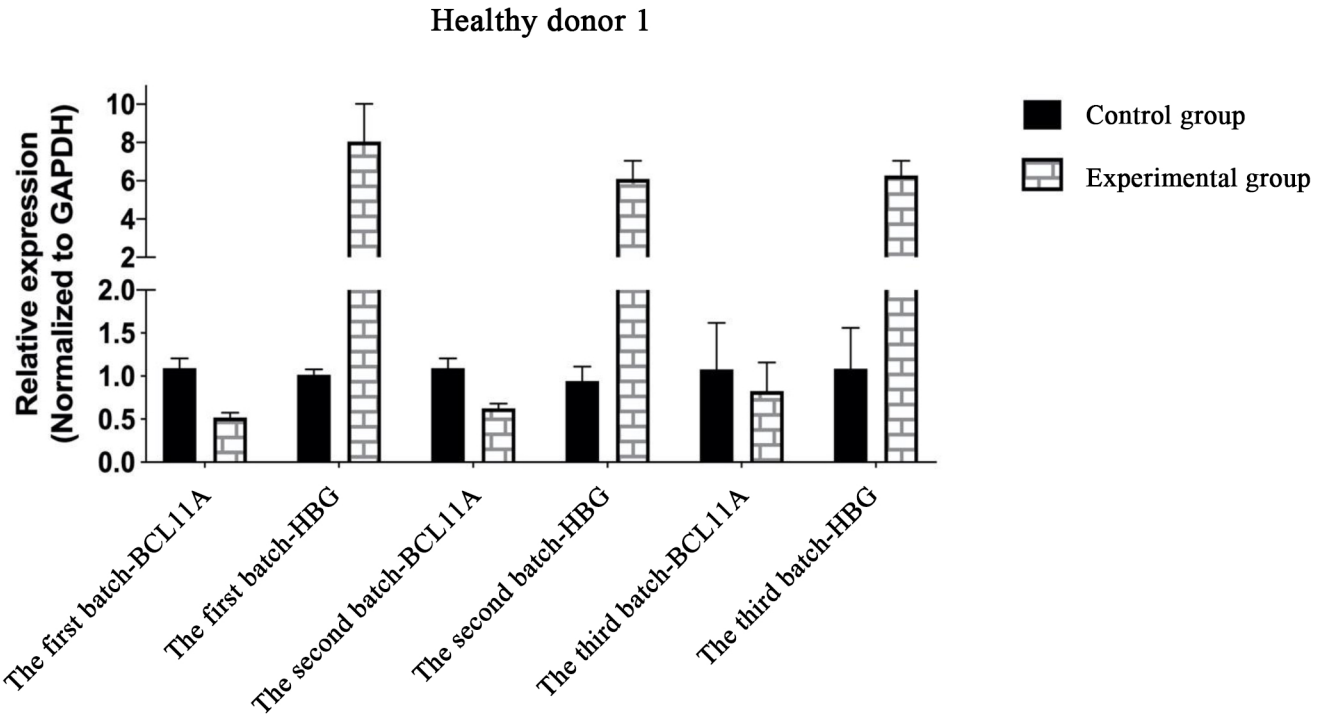

Healthy donor 1
Relative expression (Normalized to GAPDH)
10
8
6
4
2
2.0
1.5
1.0
0.5
0.0
Control group
Experimental group
The first batch-BCL11A
The first batch-HBG
The second batch-BCL11A
The second batch-HBG
The third batch-BCL11A
The third batch-HBG

Fig. 7

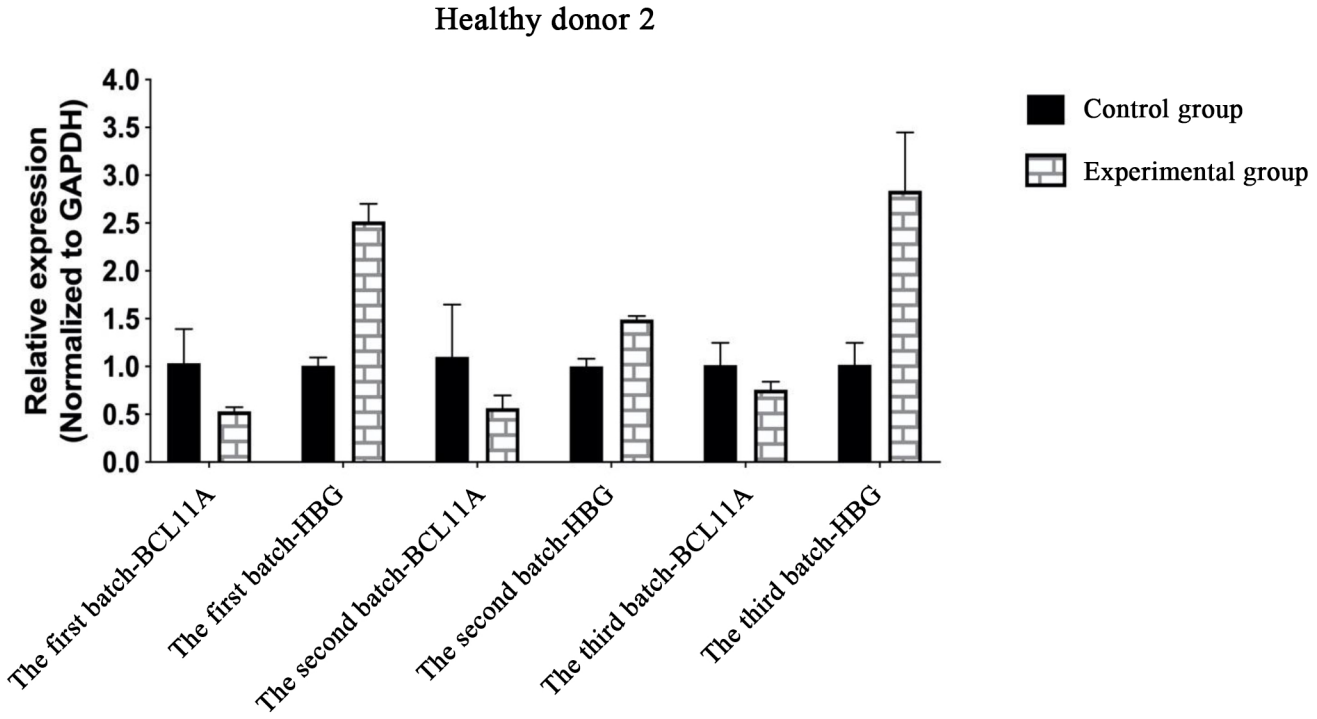

Healthy donor 2
Relative expression (Normalized to GAPDH)
4.0
3.5
3.0
2.5
2.0
1.5
1.0
0.5
0.0
Control group
Experimental group
The first batch-BCL11A
The first batch-HBG
The second batch-BCL11A
The second batch-HBG
The third batch-BCL11A
The third batch-HBG

Fig. 8

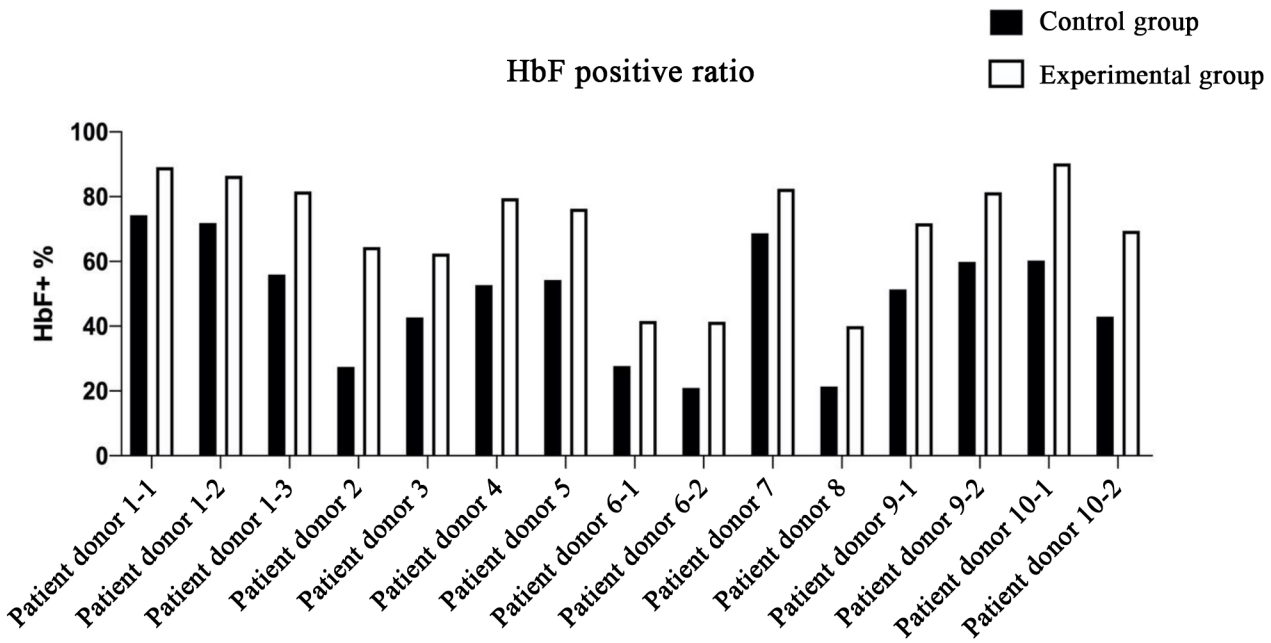

Control group
Experimental group
HbF positive ratio
HbF+ %
100
80
60
40
20
0
Patient donor 1-1
Patient donor 1-2
Patient donor 1-3
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Patient donor 6-1
Patient donor 6-2
Patient donor 7
Patient donor 8
Patient donor 9-1
Patient donor 9-2
Patient donor 10-1
Patient donor 10-2

Fig. 9A

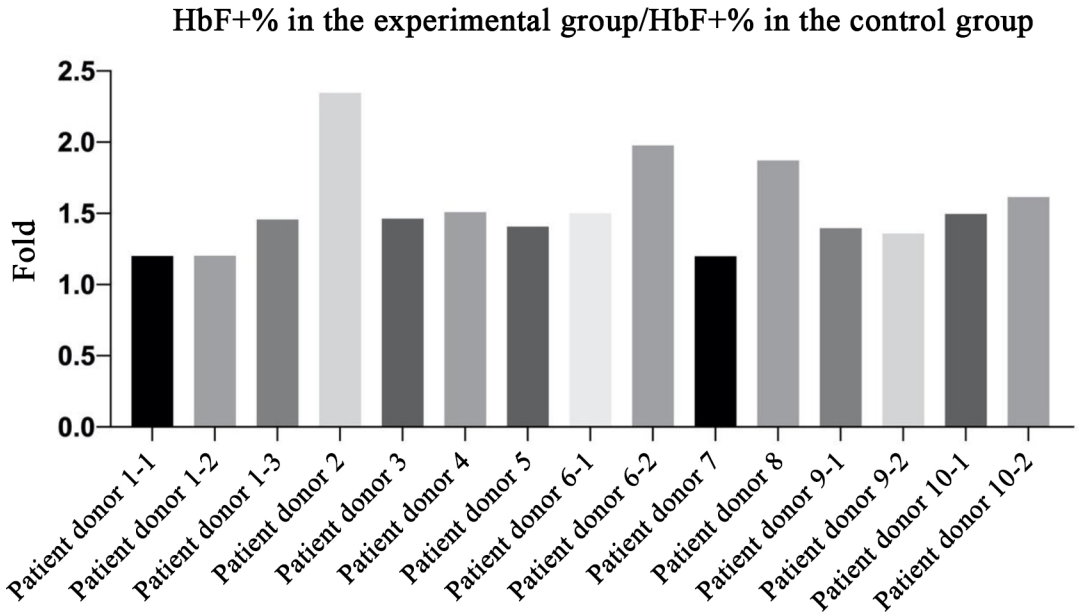

HbF+% in the experimental group/HbF+% in the control group
Fold
2.5
2.0
1.5
1.0
0.5
0.0
Patient donor 1-1
Patient donor 1-2
Patient donor 1-3
Patient donor 2
Patient donor 3
Patient donor 4
Patient donor 5
Patient donor 6-1
Patient donor 6-2
Patient donor 7
Patient donor 8
Patient donor 9-1
Patient donor 9-2
Patient donor 10-1
Patient donor 10-2

Fig. 9B

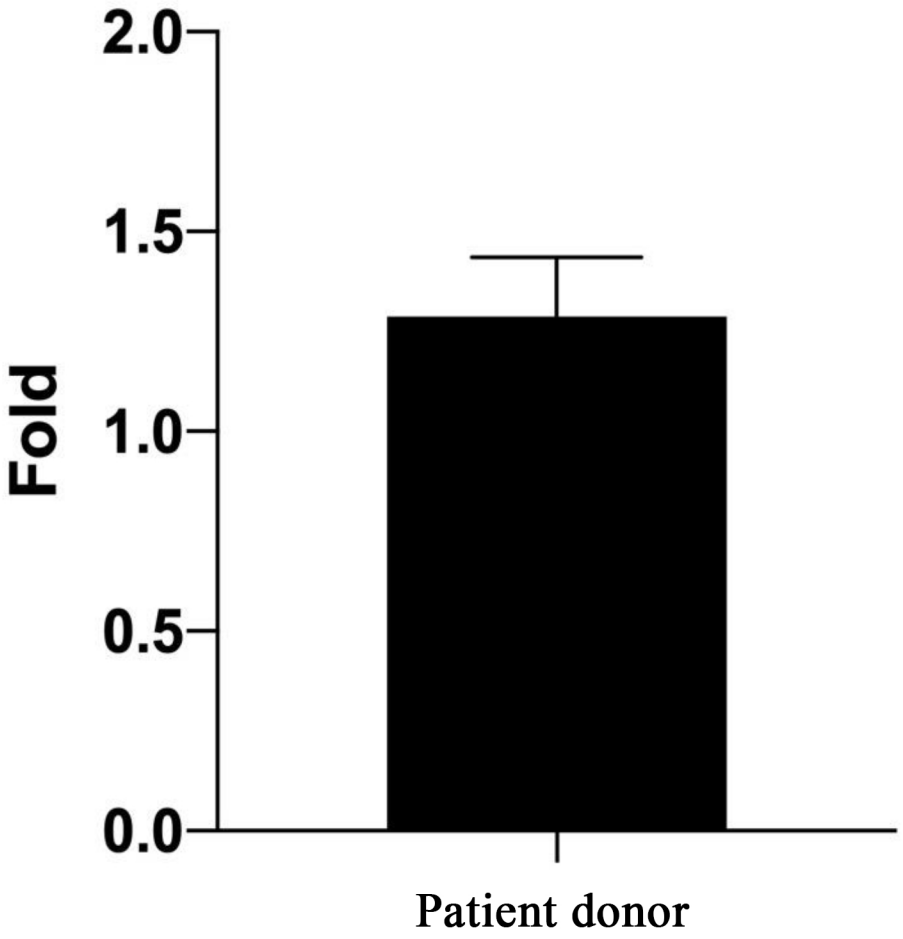


Fig. 10

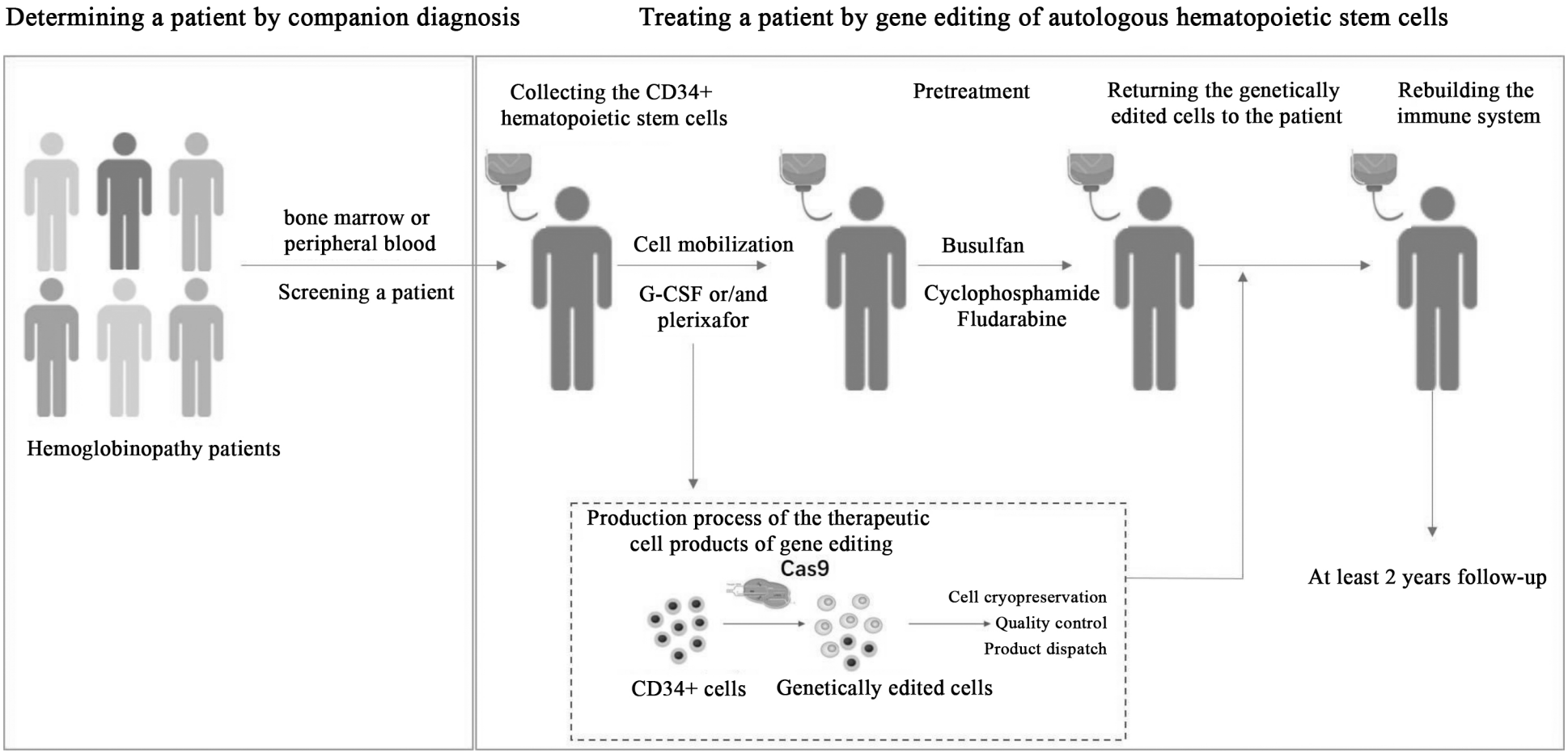

Determining a patient by companion diagnosis
Treating a patient by gene editing of autologous hematopoietic stem cells
Collecting the CD34+ hematopoietic stem cells
Pretreatment
Returning the genetically edited cells to the patient
Rebuilding the immune system
bone marrow or peripheral blood
Screening a patient
Hemoglobinopathy patients
Cell mobilization
G-CSF or/and plerixafor
Busulfan
Cyclophosphamide
Fludarabine
Production process of the therapeutic cell products of gene editing
Cas9
Cell cryopreservation
Quality control
Product dispatch
CD34+ cells
Genetically edited cells
At least 2 years follow-up

Fig. 11