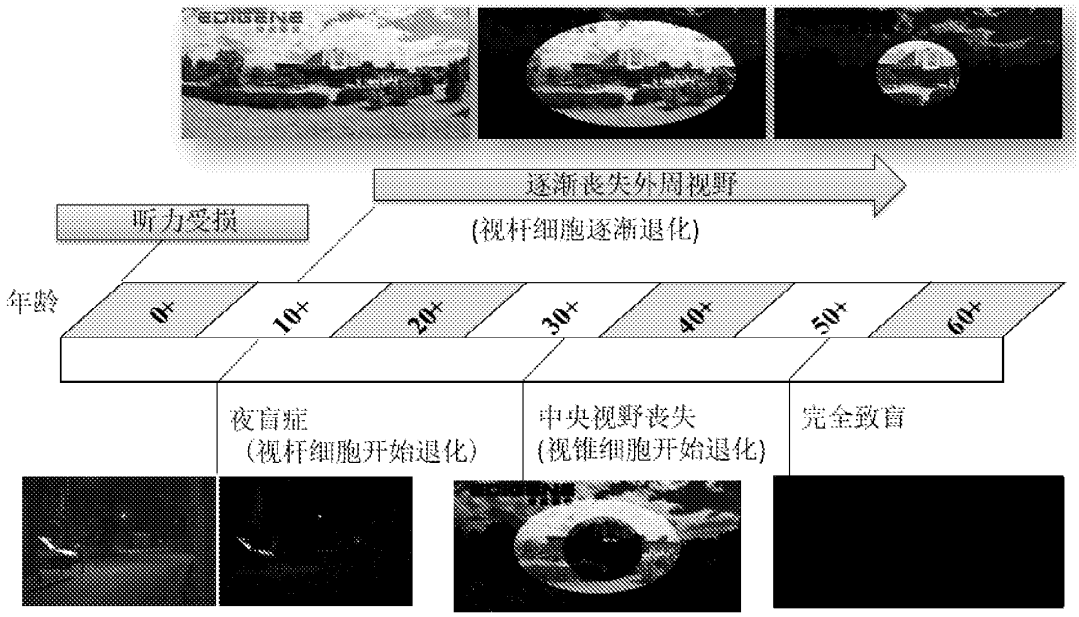
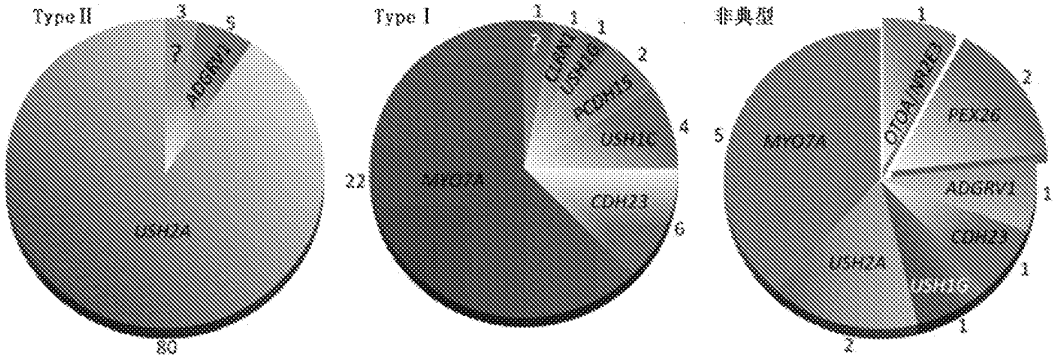

逐渐丧失外周视野
(视杆细胞逐渐退化)
听力受损
年龄
0+
10+
20+
30+
40+
50+
60+
夜盲症
(视杆细胞开始退化)
中央视野丧失
(视锥细胞开始退化)
完全致盲

图 1



USH2A中的突变：91%的II型Usher(80/88)
全部Usher的59%(82/138)

图 2

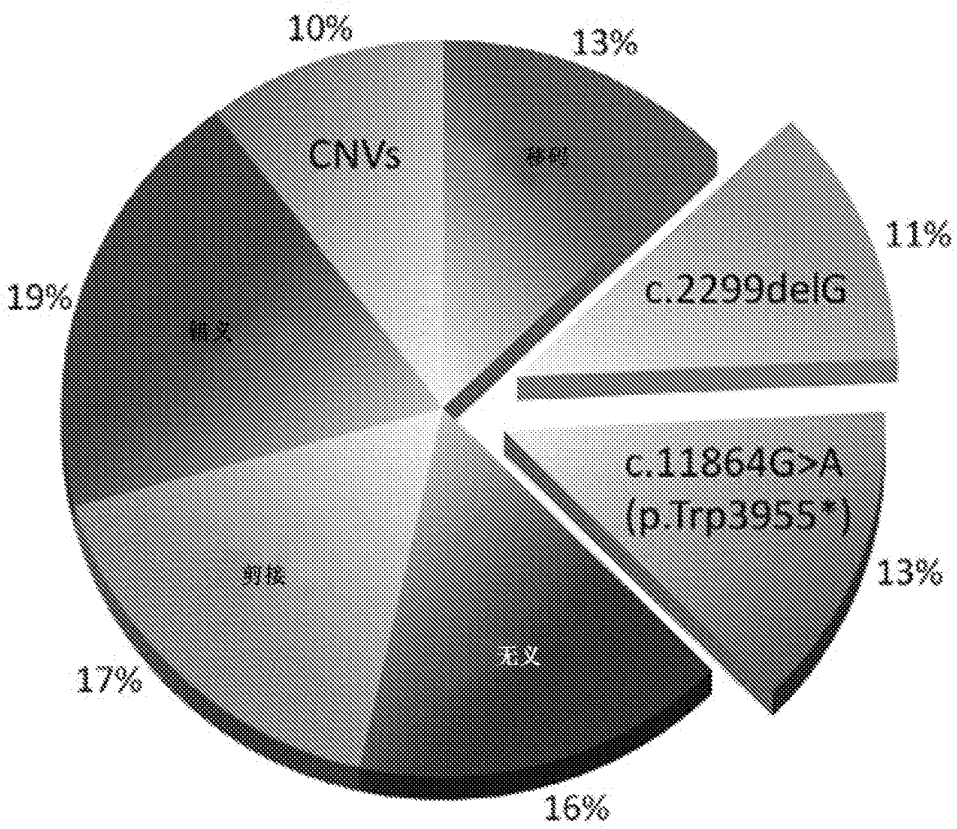


图 3

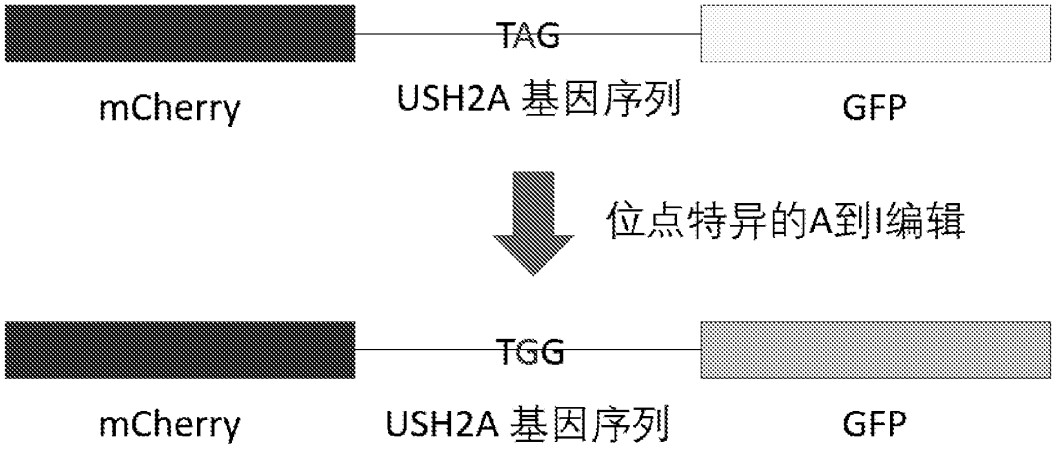

TAG
mCherry
USH2A 基因序列
GFP
位点特异的A到I编辑
TGG
mCherry
USH2A 基因序列
GFP

图 4

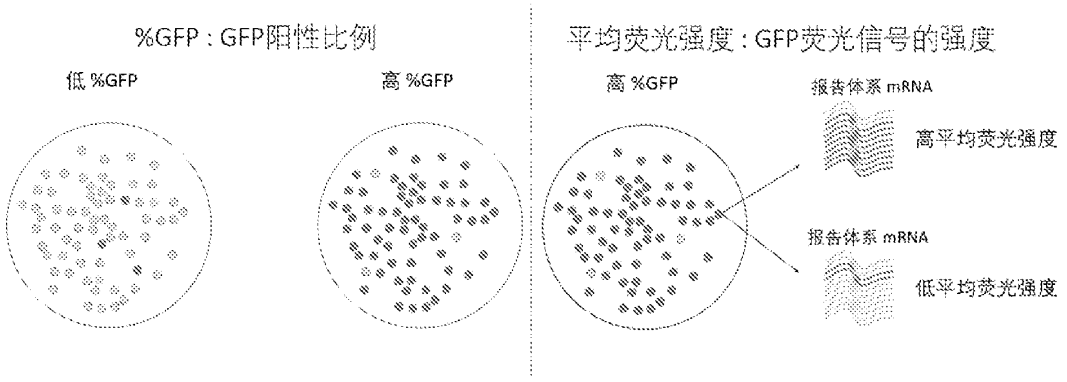

%GFP：GFP阳性比例
低 %GFP
高 %GFP
平均荧光强度：GFP荧光信号的强度
高 %GFP
报告体系 mRNA
高平均荧光强度
报告体系 mRNA
低平均荧光强度

图 5

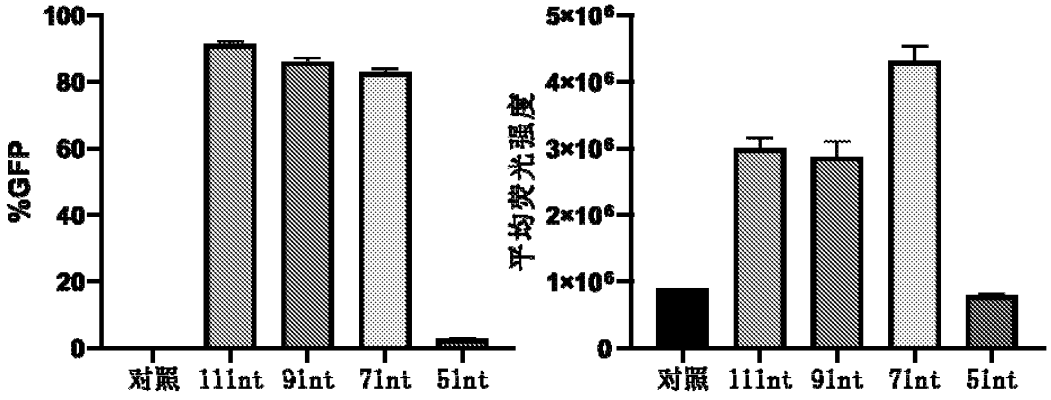

%GFP
100
80
60
40
20
0
对照
111nt
91nt
71nt
51nt
平均荧光强度
5×10⁶
4×10⁶
3×10⁶
2×10⁶
1×10⁶
0
对照
111nt
91nt
71nt
51nt

图 6

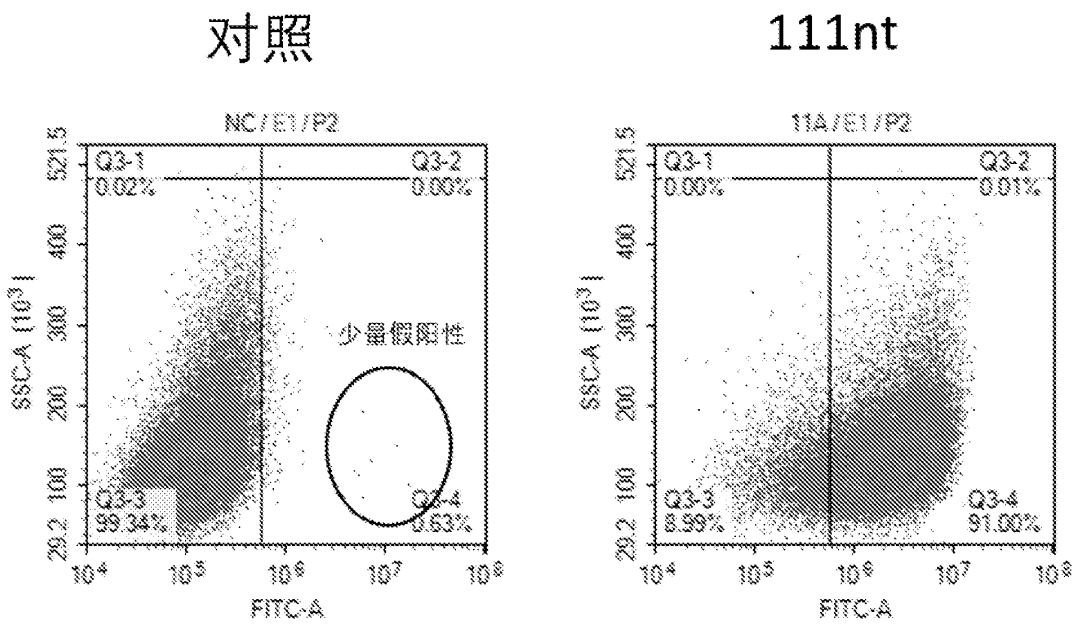

对照
111nt
NC/E1/P2
11A/E1/P2
Q3-1
0.02%
Q3-2
0.00%
Q3-3
99.34%
Q3-4
0.63%
少量假阳性
Q3-1
0.00%
Q3-2
0.01%
Q3-3
8.99%
Q3-4
91.00%
SSC-A (10³)
FITC-A

图 7

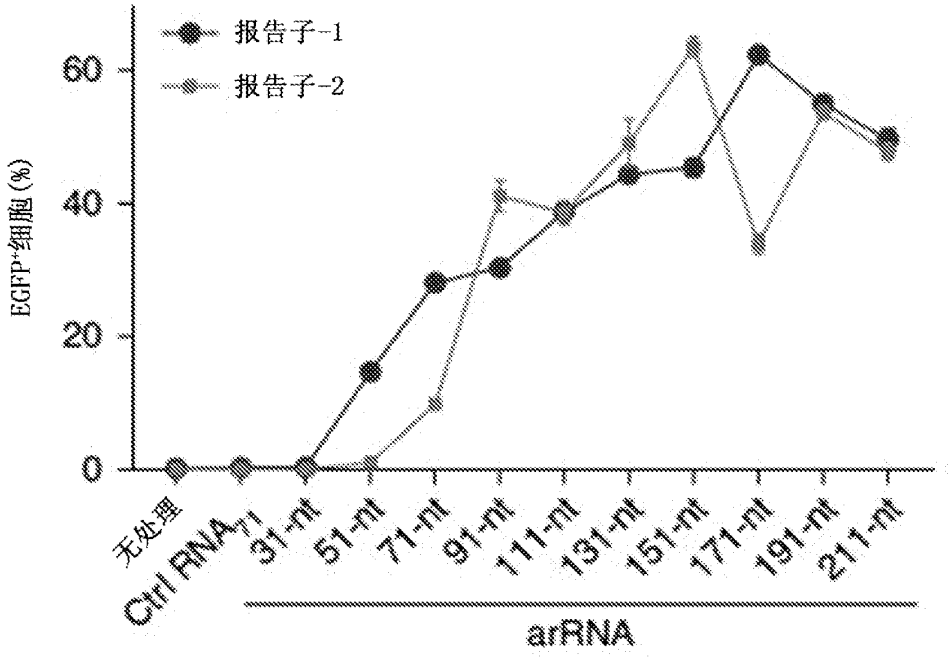

报告子-1
报告子-2
EGFP+细胞 (%)
无处理
Ctrl RNA
31-nt
51-nt
71-nt
91-nt
111-nt
131-nt
151-nt
171-nt
191-nt
211-nt
arRNA

图 8

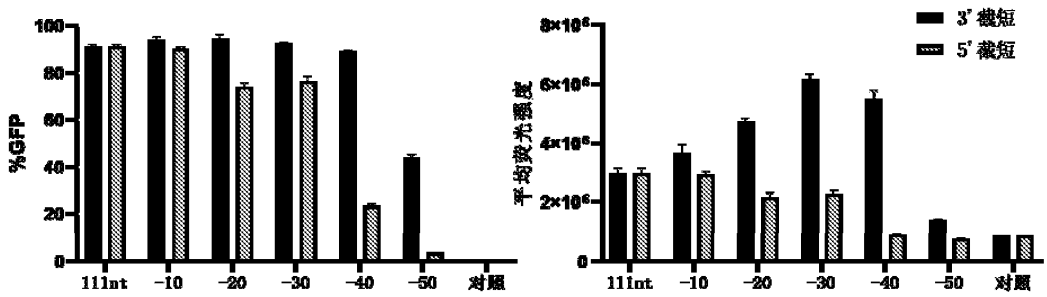

%GFP
100
80
60
40
20
0
111nt
-10
-20
-30
-40
-50
对照
平均荧光强度
8x10⁶
6x10⁶
4x10⁶
2x10⁶
0
3'截短
5'截短

图 9

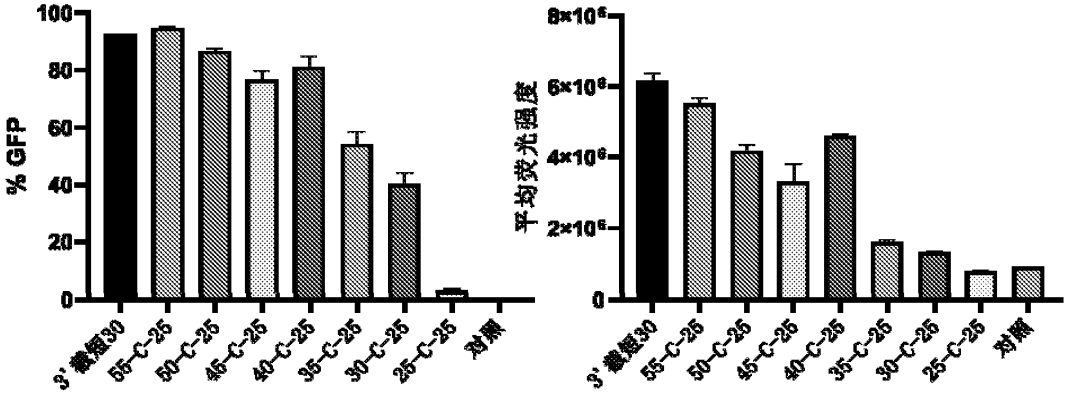

% GFP
100
80
60
40
20
0
3'截短30
55-C-25
50-C-25
45-C-25
40-C-25
35-C-25
30-C-25
25-C-25
对照
平均荧光强度
8x10⁶
6x10⁶
4x10⁶
2x10⁶
0

图 10

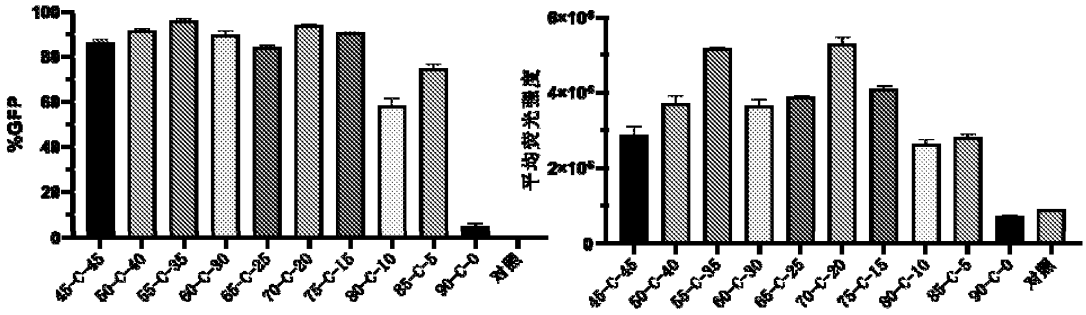

%GFP
平均荧光强度
45-C-45
50-C-40
55-C-35
60-C-30
65-C-25
70-C-20
75-C-15
80-C-10
85-C-5
90-C-0
对照

图 11

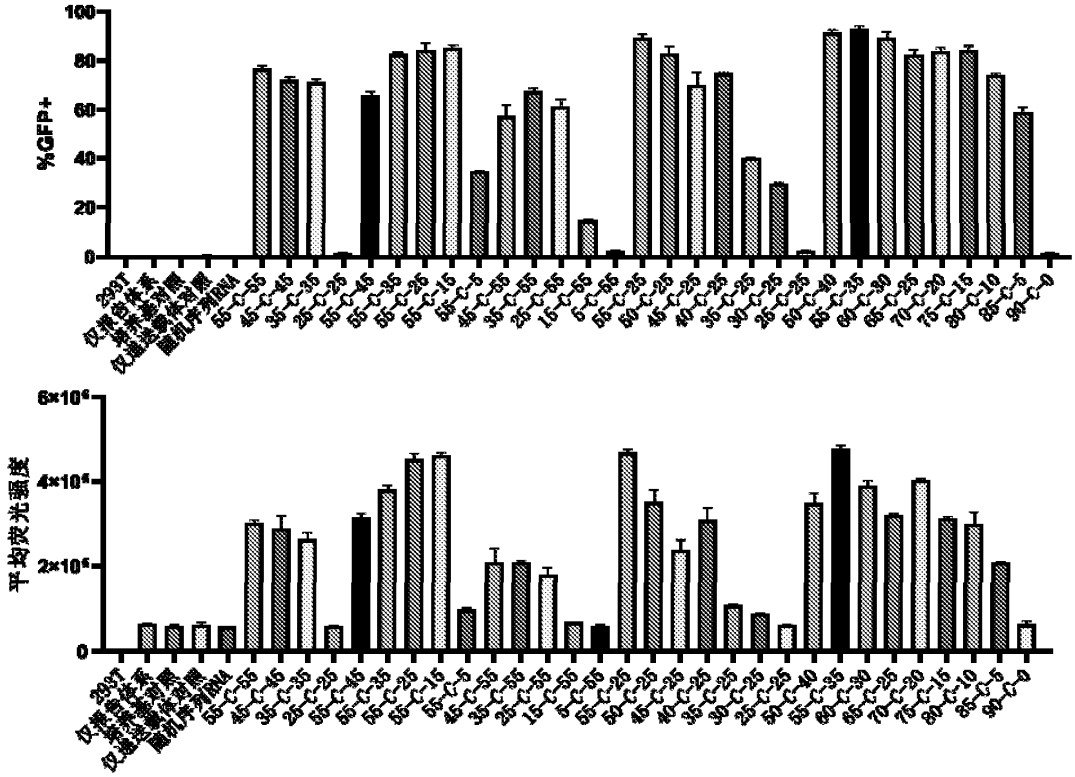

%GFP+
平均荧光强度
293T
仅报告体系
转序基对照
仅递送载体对照
随机序列RNA
55-C-55
45-C-45
35-C-35
25-C-25
55-C-45
55-C-35
55-C-25
55-C-15
55-C-5
45-C-55
35-C-55
25-C-55
15-C-55
5-C-55
55-C-25
50-C-25
45-C-25
40-C-25
35-C-25
30-C-25
25-C-25
50-C-40
55-C-35
60-C-30
65-C-25
70-C-20
75-C-15
80-C-10
85-C-5
90-C-0

图 12

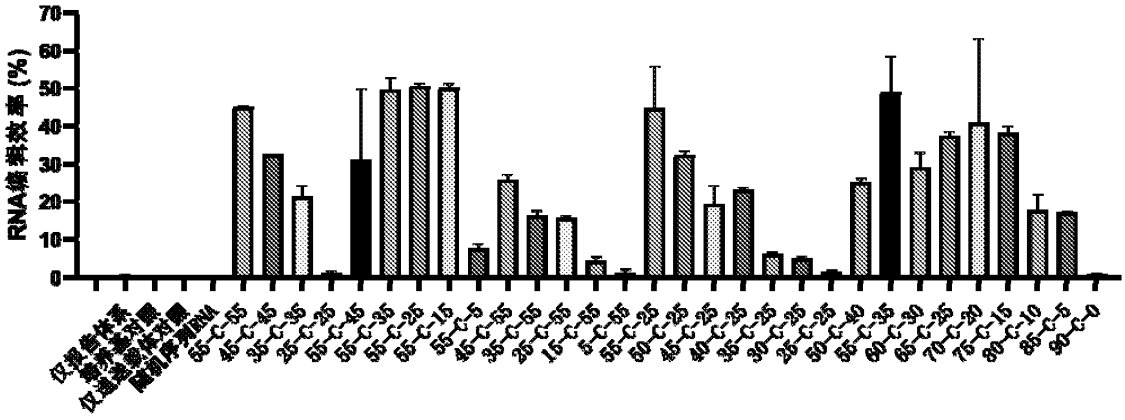

RNA编辑效率 (%)
70
60
50
40
30
20
10
0
55-C-55
45-C-45
35-C-35
25-C-25
55-C-45
55-C-35
55-C-25
55-C-15
55-C-5
45-C-55
35-C-55
25-C-55
15-C-55
5-C-55
55-C-25
50-C-25
45-C-25
40-C-25
35-C-25
30-C-25
25-C-25
50-C-40
55-C-35
60-C-30
65-C-25
70-C-20
75-C-15
80-C-10
85-C-5
90-C-0

图 13

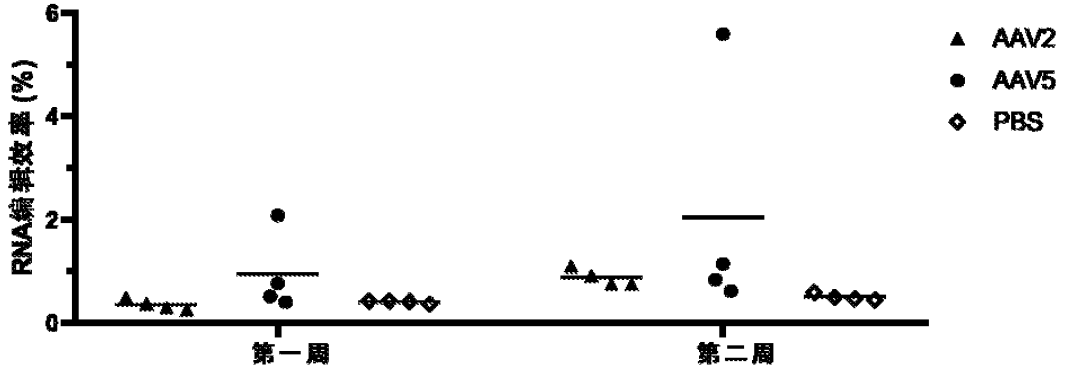

RNA编辑效率 (%)
6
4
2
0
第一周
第二周
AAV2
AAV5
PBS

图 14

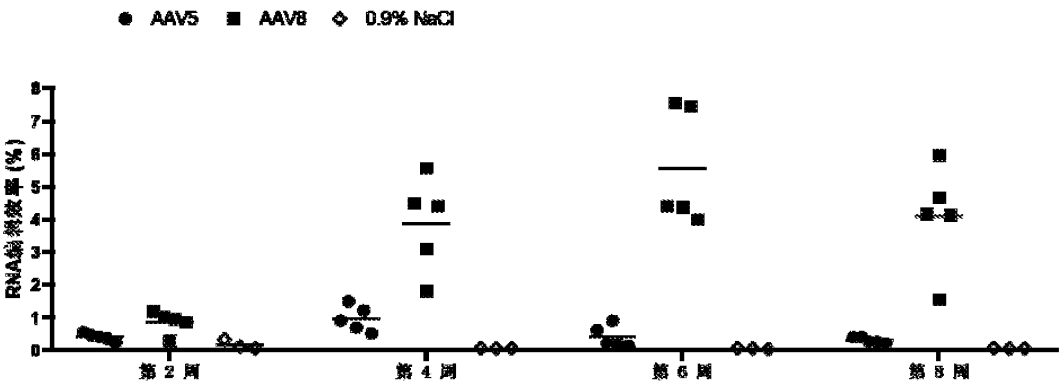


图 15