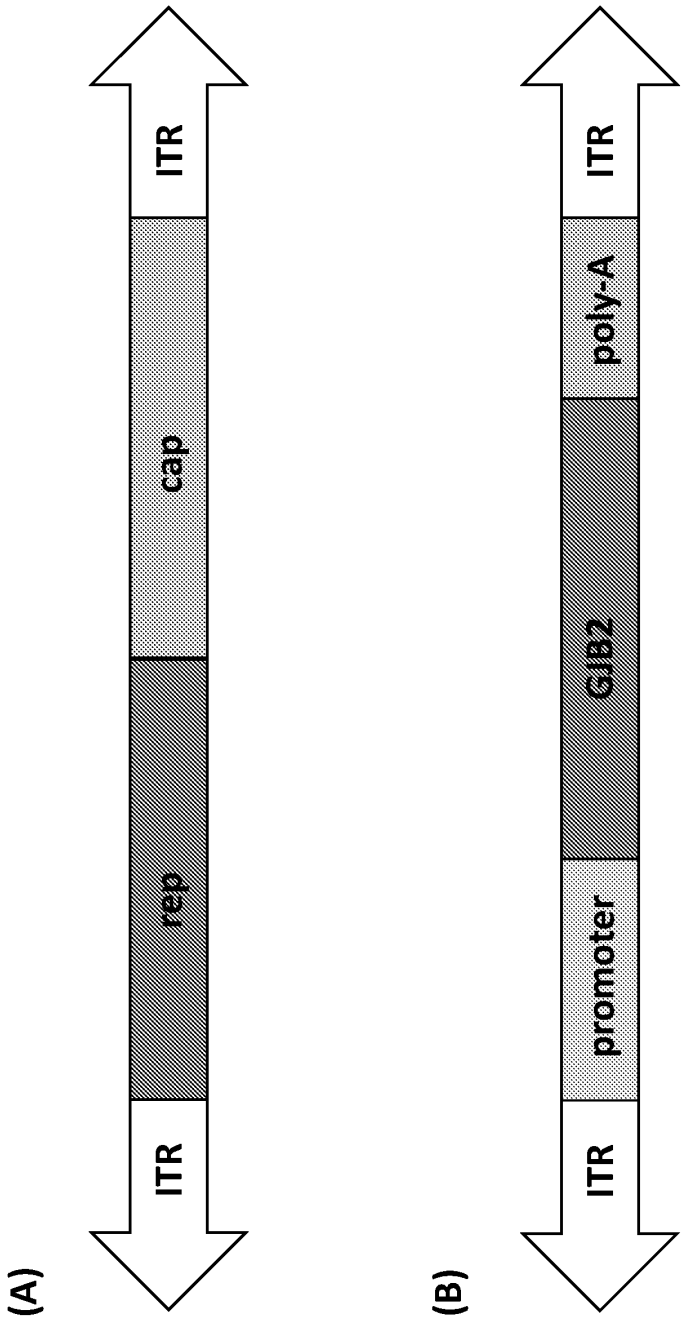

FIG. 1

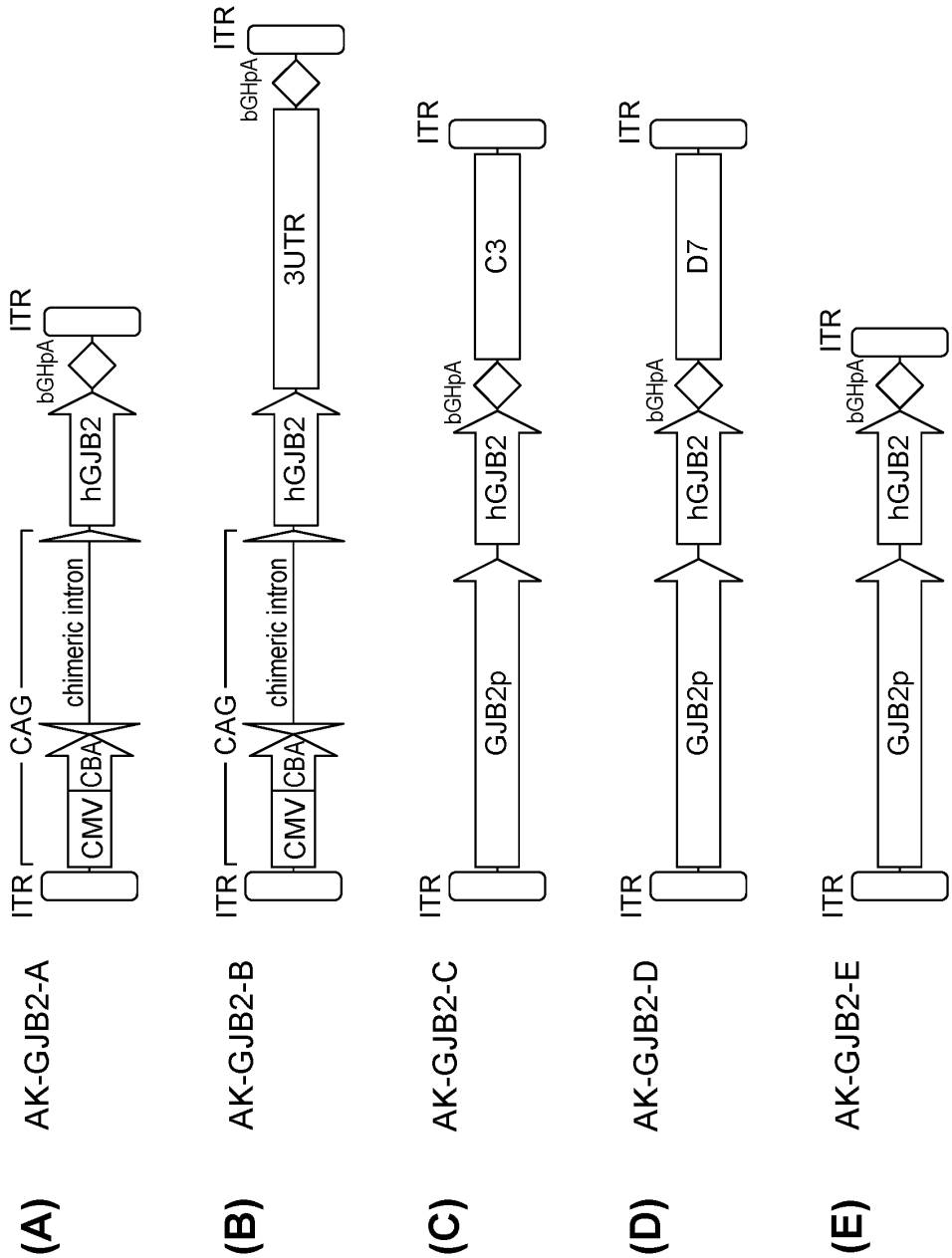
(A)
AK-GJB2-A
ITR
CAG
CMV
CBA
chimeric intron
hGJB2
bGHpA
ITR
(B)
AK-GJB2-B
ITR
CAG
CMV
CBA
chimeric intron
hGJB2
3UTR
bGHpA
ITR
(C)
AK-GJB2-C
ITR
GJB2p
hGJB2
C3
bGHpA
ITR
(D)
AK-GJB2-D
ITR
GJB2p
hGJB2
D7
bGHpA
ITR
(E)
AK-GJB2-E
ITR
GJB2p
hGJB2
bGHpA
ITR

FIG. 2

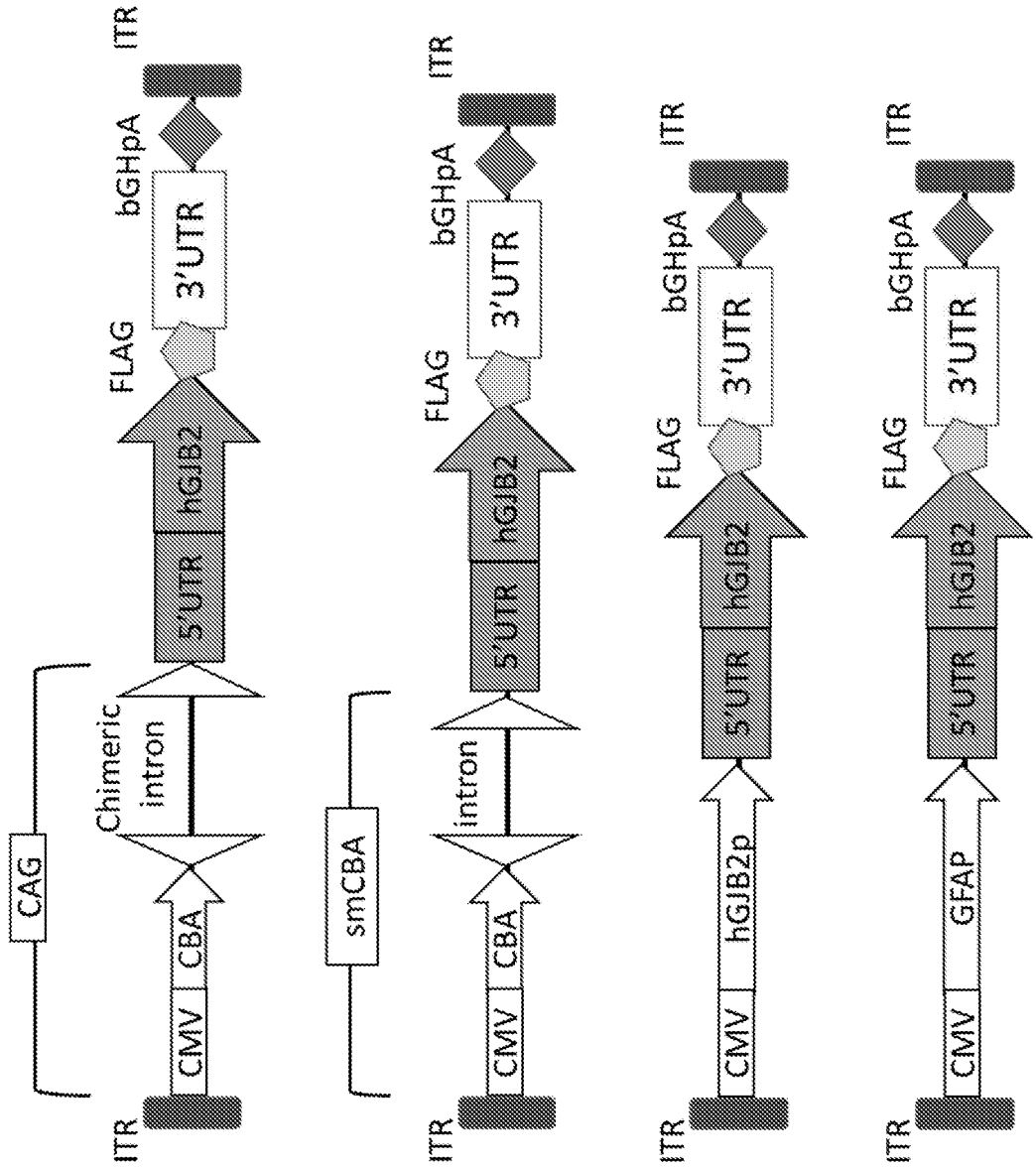

FIG. 2 Continued

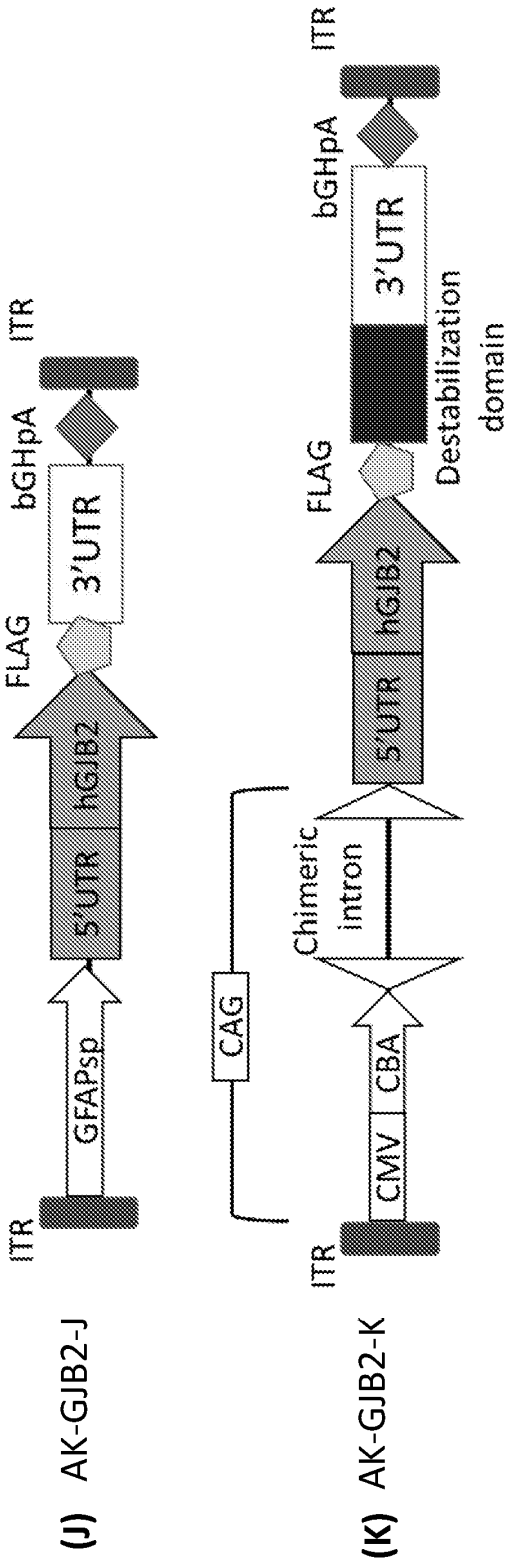

FIG. 2 Continued

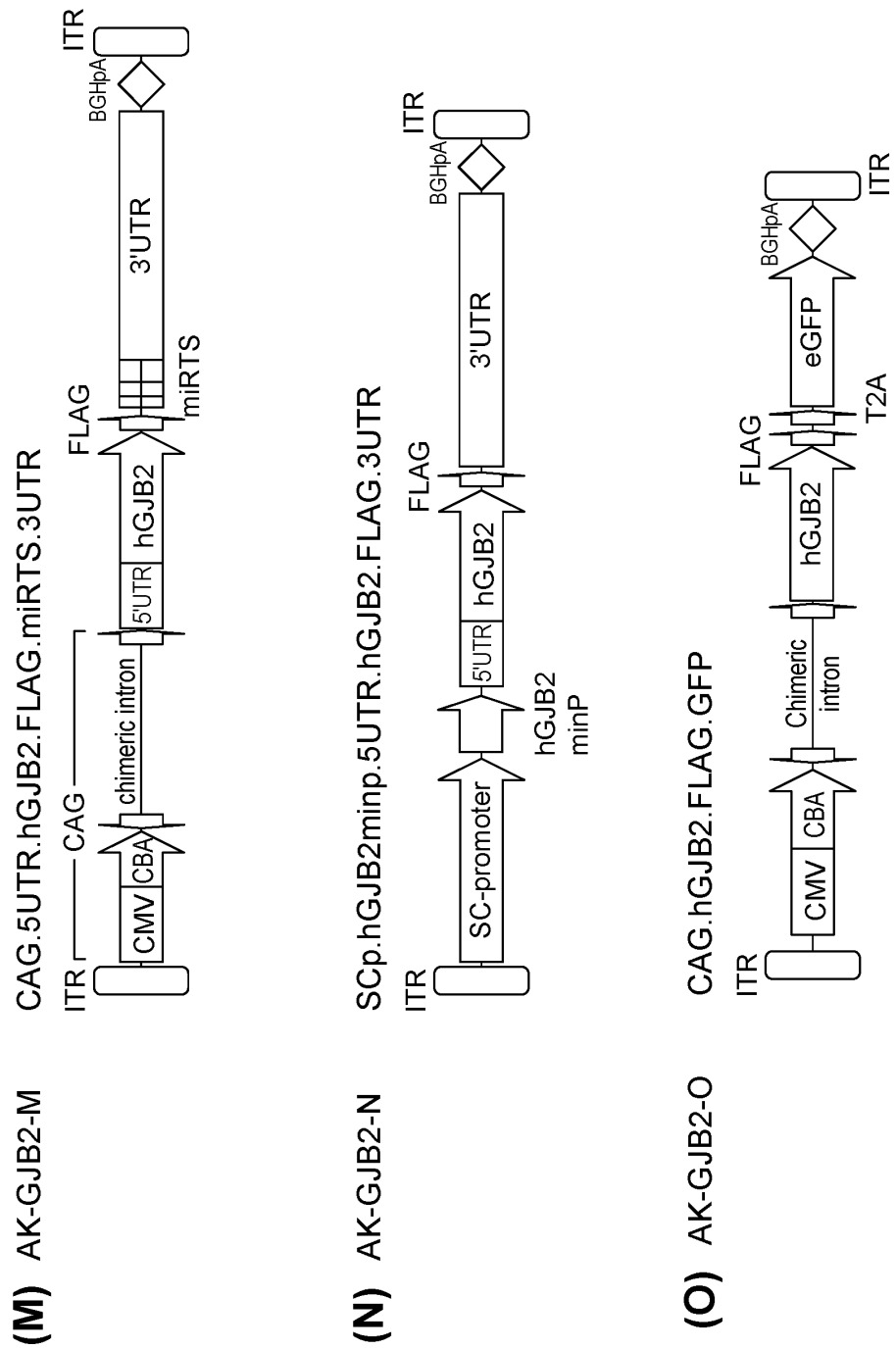

FIG. 2 Continued

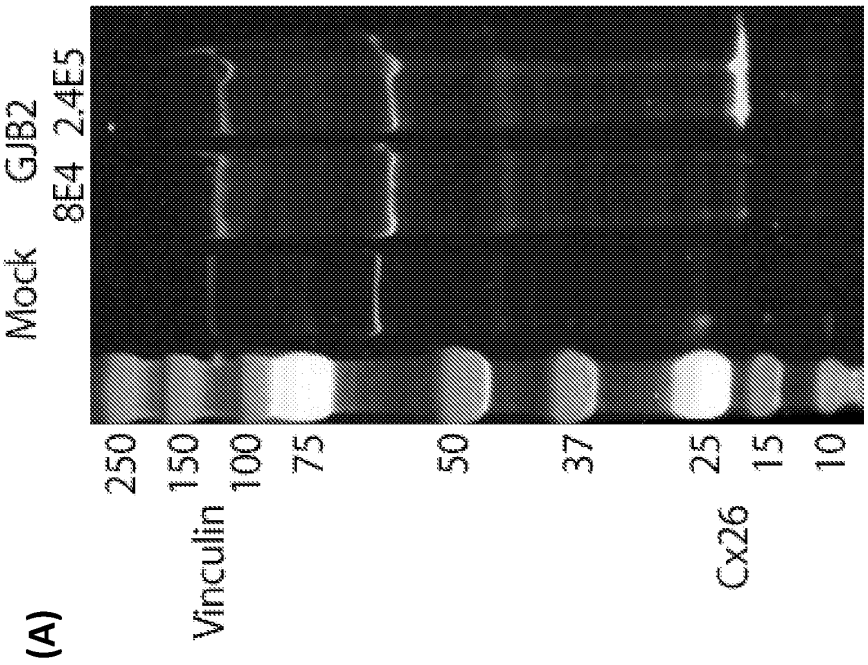

FIG. 3

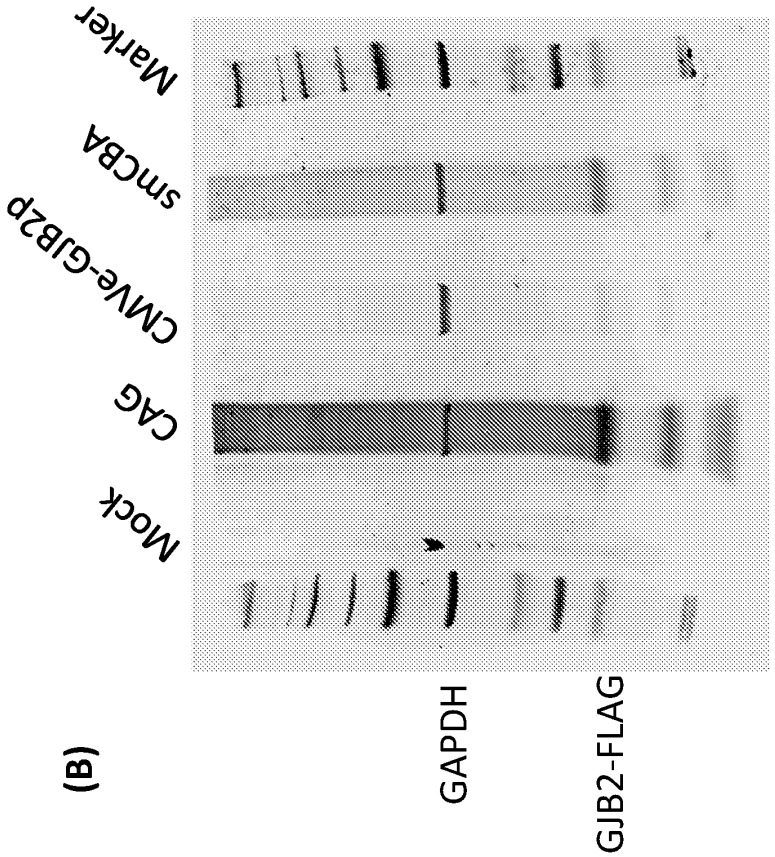

FIG. 3

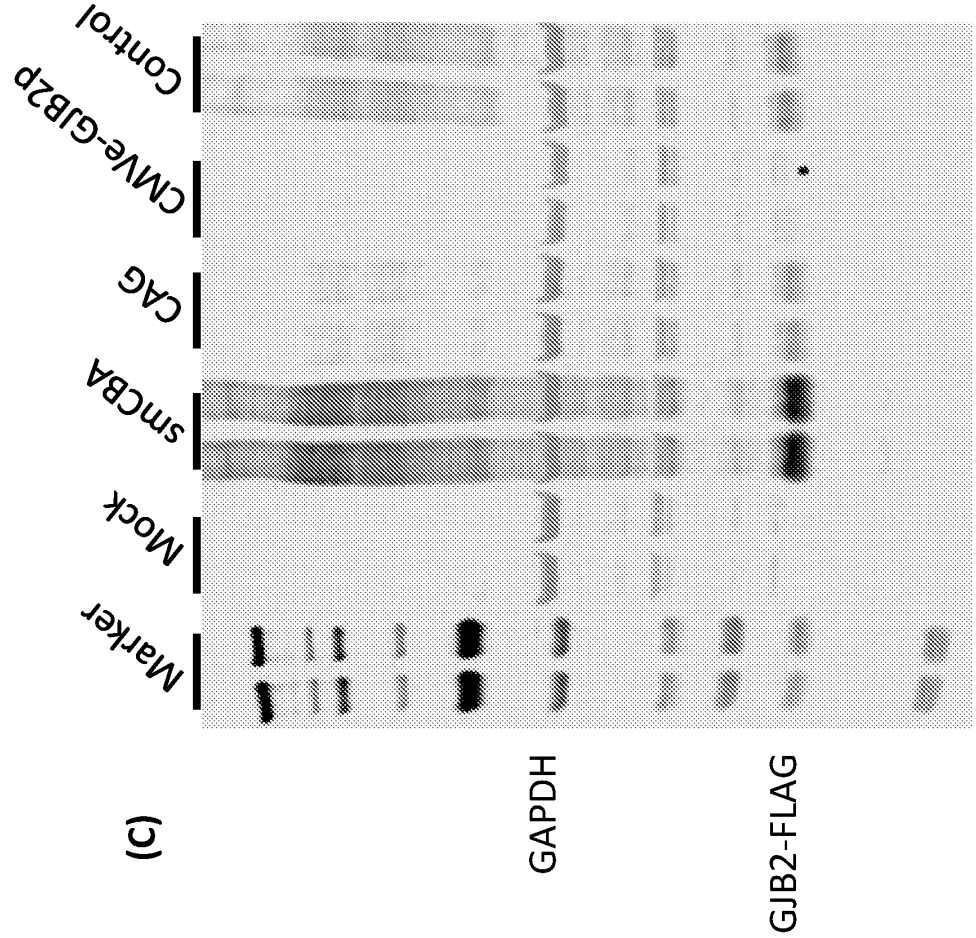

FIG. 3

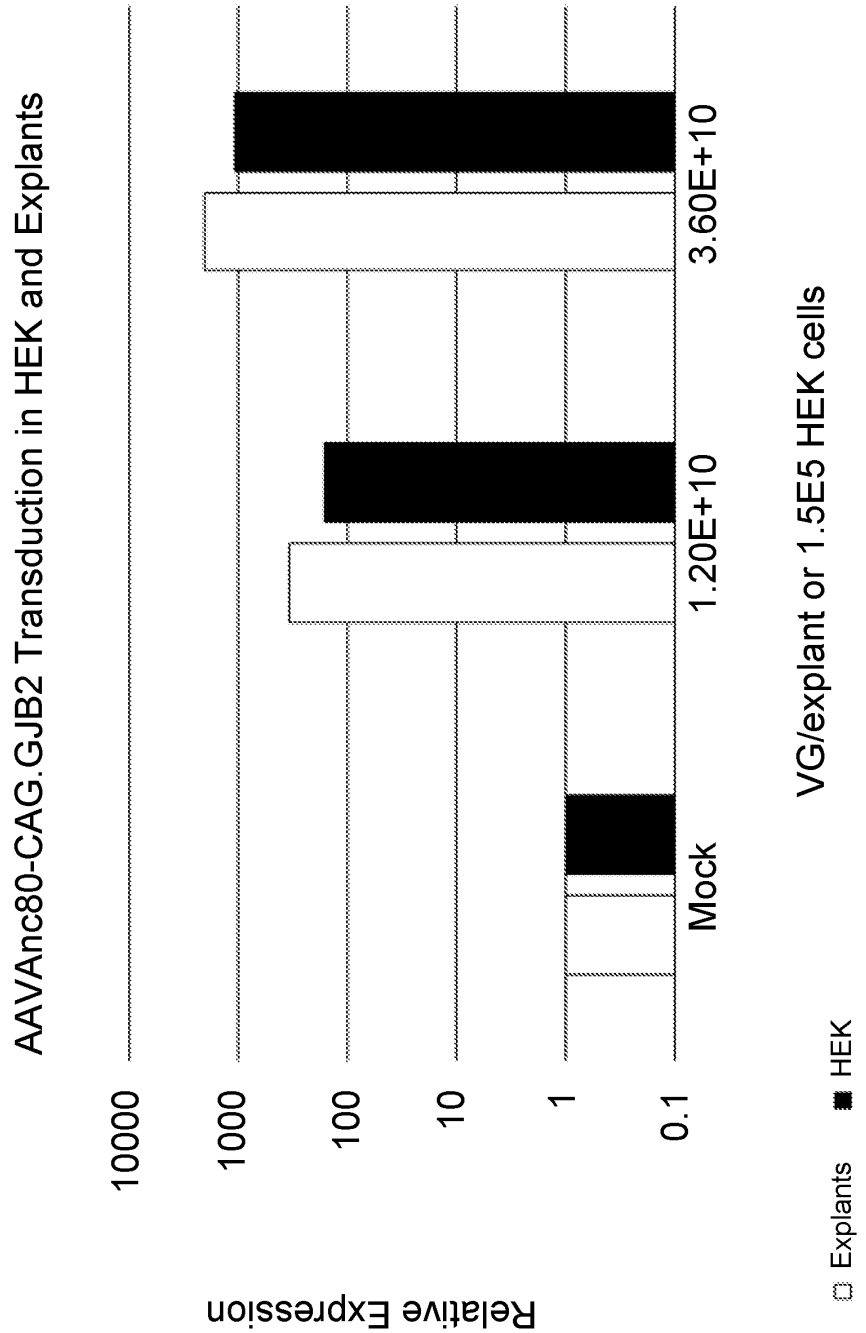
AAVAnc80-CAG.GJB2 Transduction in HEK and Explants
Relative Expression
10000
1000
100
10
1
0.1
Mock
1.20E+10
3.60E+10
VG/explant or 1.5E5 HEK cells
Explants
HEK

FIG. 4

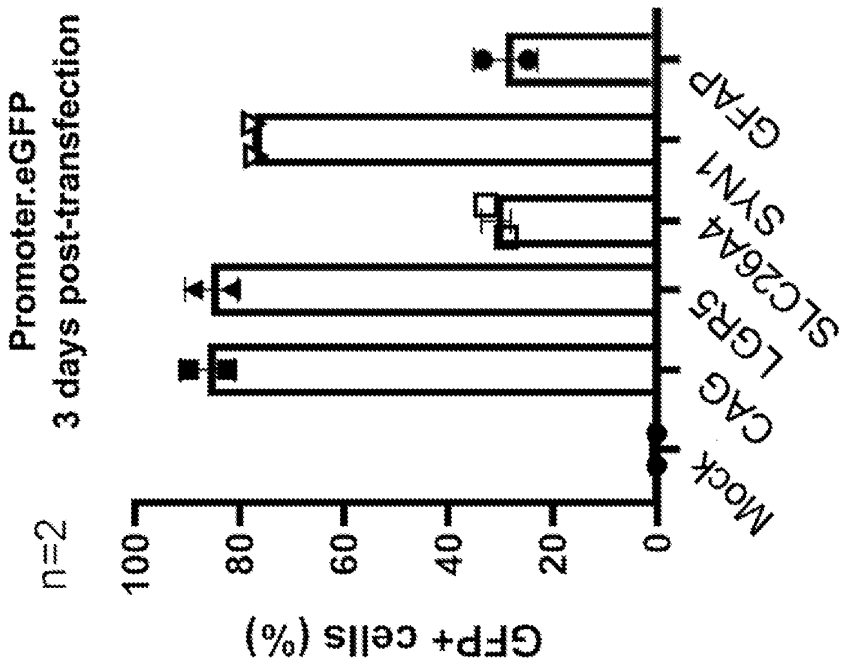
(A)
Promoter.eGFP
3 days post-transfection
n=2
GFP+ cells (%)
100
80
60
40
20
0
Mock
CAG
LGR5
SLC26A4
SYN1
GFAP

FIG. 5

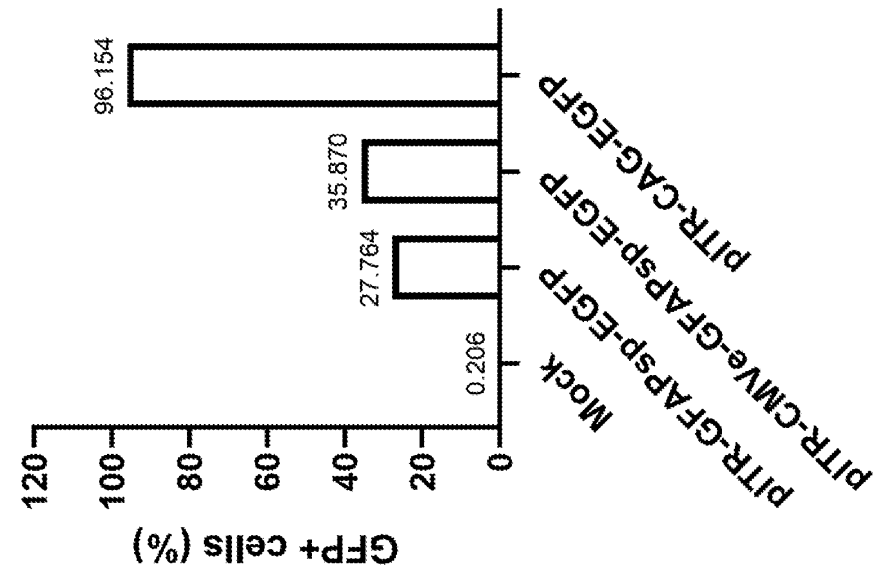
(B)
GFP+ cells (%)
120
100
80
60
40
20
0
96.154
35.870
27.764
0.206
Mock
pITR-GFAPsp-EGFP
pITR-CMVe-GFAPsp-EGFP
pITR-CAG-EGFP

FIG. 5

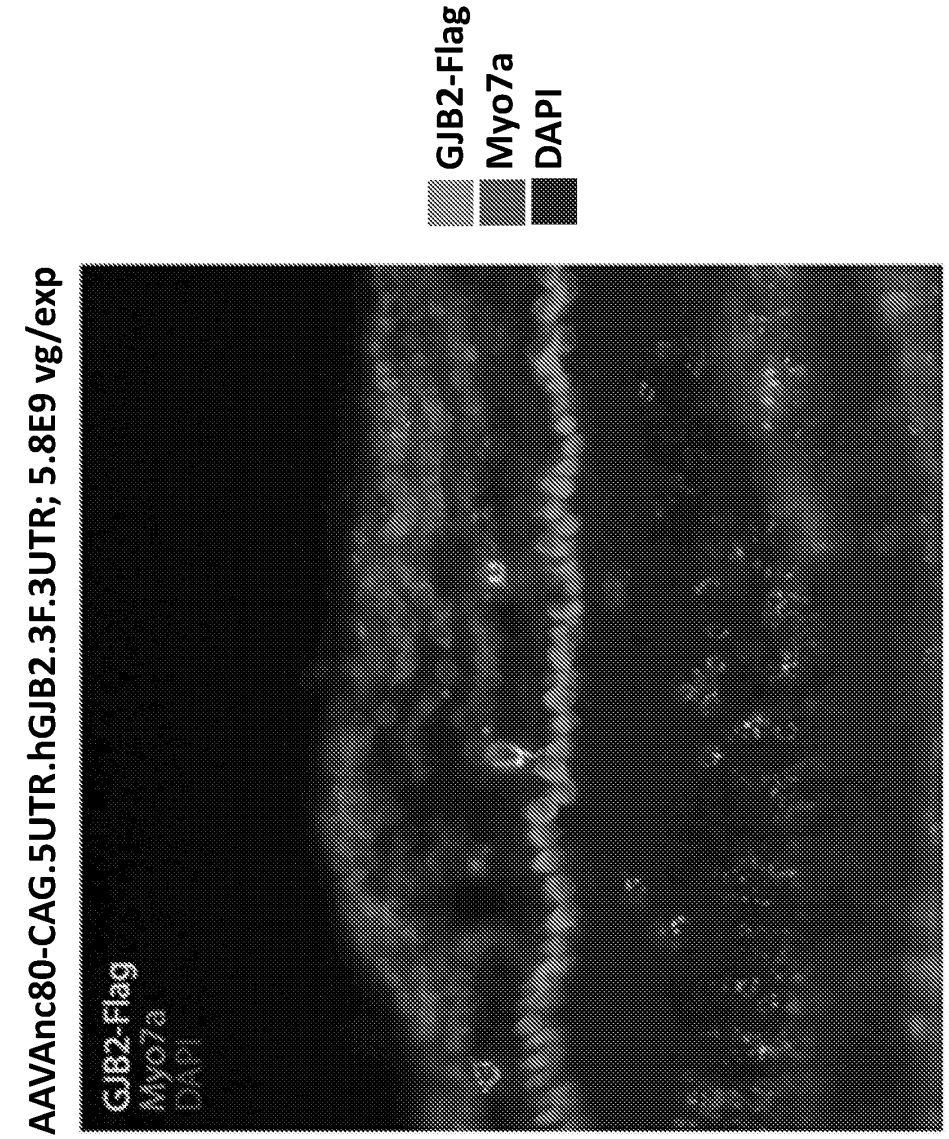
(A)
AAVAnc80-CAG.5UTR.hGJB2.3F.3UTR; 5.8E9 vg/exp
GJB2-Flag
Myo7a
DAPI
GJB2-Flag
Myo7a
DAPI

FIG. 6

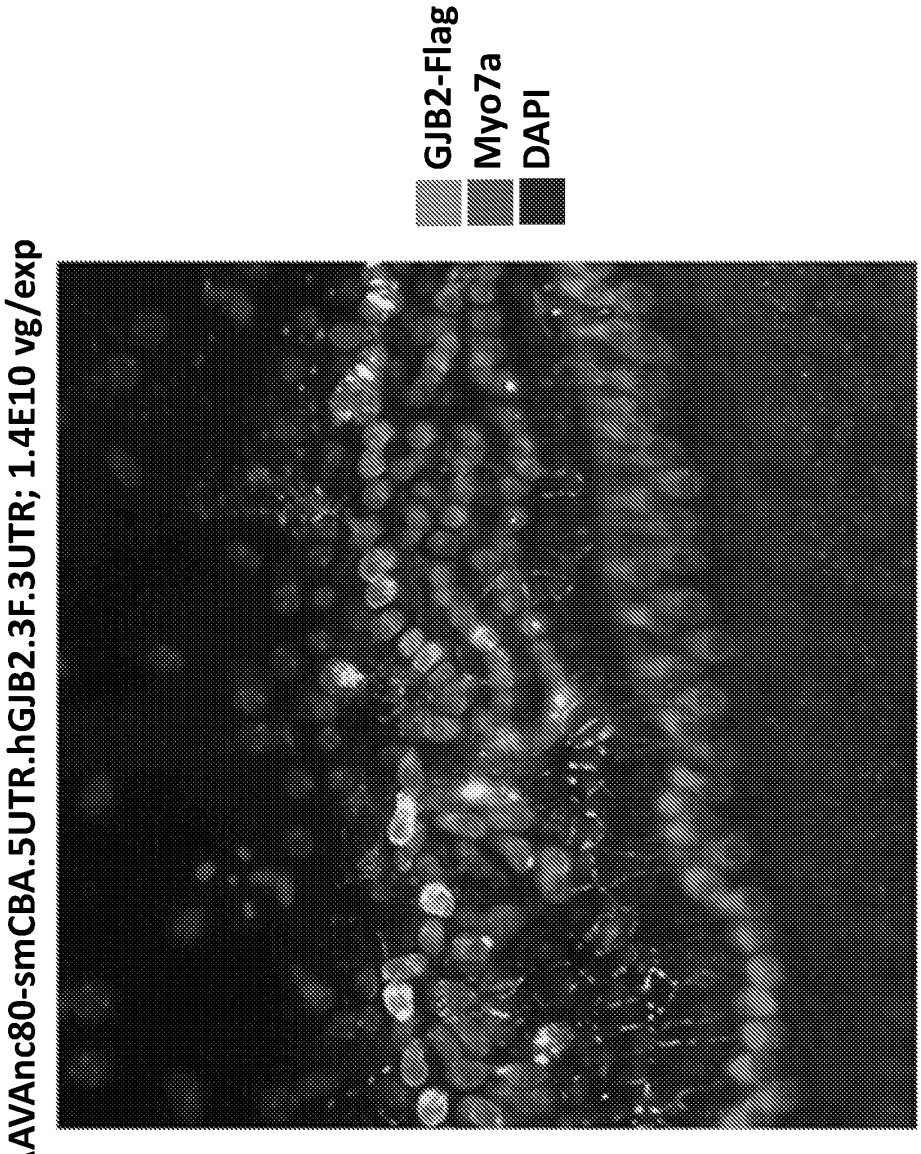
(B) AAVAnc80-smCBA.5UTR.hGJB2.3F.3UTR; 1.4E10 vg/exp
GJB2-Flag
Myo7a
DAPI

FIG. 6

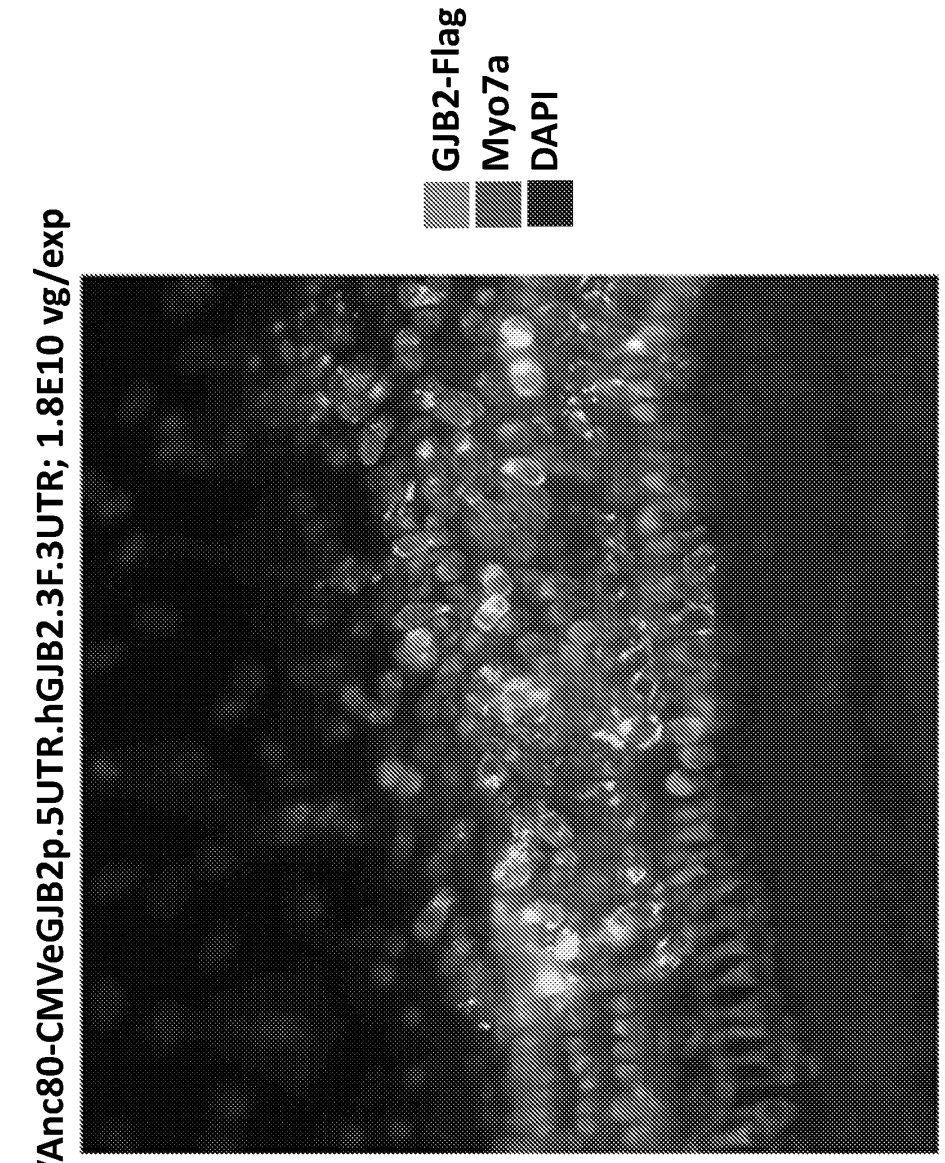
(C) AAVAnc80-CMVeGJB2p.5UTR.hGJB2.3F.3UTR; 1.8E10 vg/exp
GJB2-Flag
Myo7a
DAPI

FIG. 6

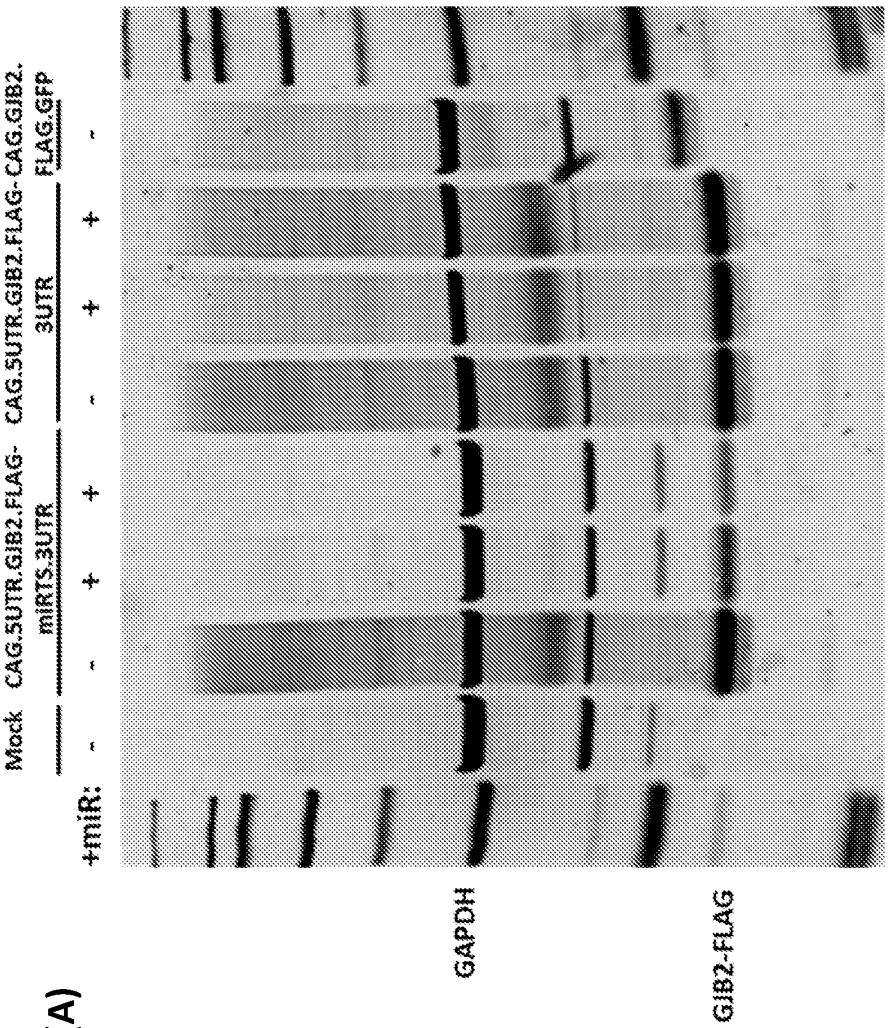
(A)
+miR:
Mock
CAG.5UTR.GIB2.FLAG-miRTS.3UTR
CAG.5UTR.GIB2.FLAG-3UTR
CAG.GIB2.FLAG.GFP
GAPDH
GIB2-FLAG

FIG. 7

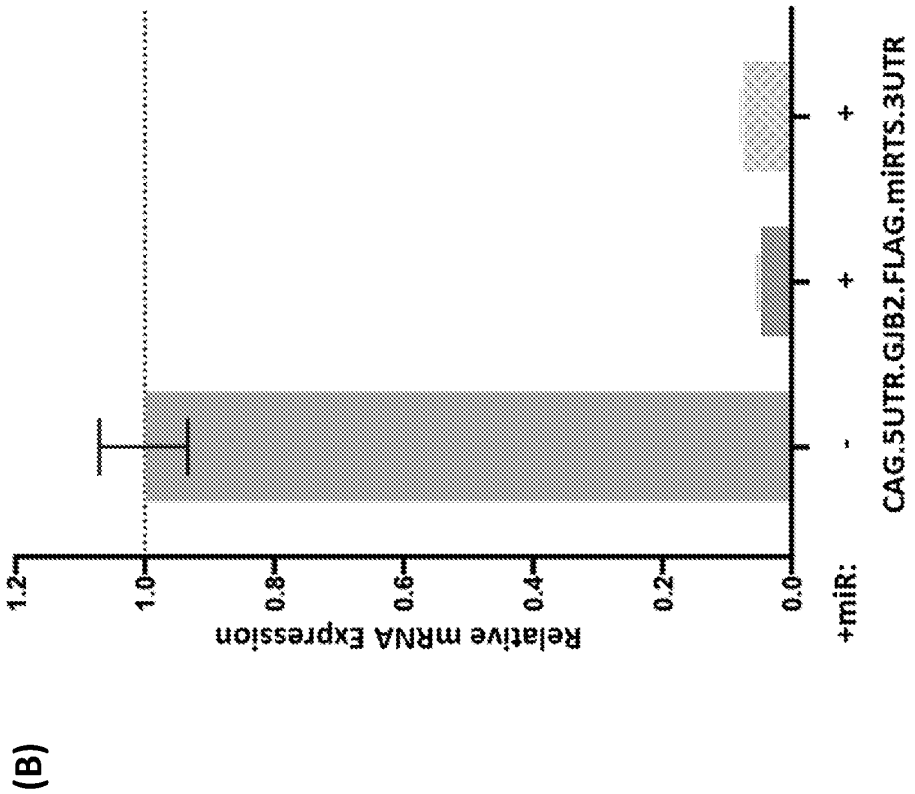
(B)
Relative mRNA Expression
1.2
1.0
0.8
0.6
0.4
0.2
0.0
+miR:
-
+
+
CAG.5UTR.GJB2.FLAG.miRTS.3UTR

FIG. 7

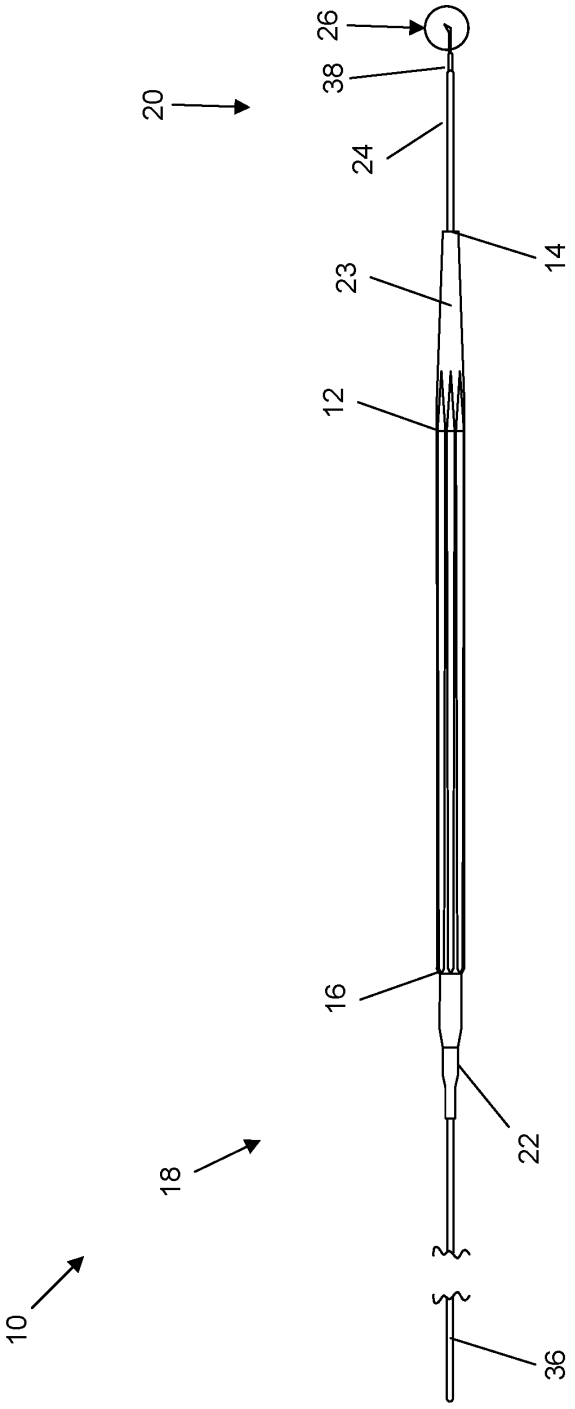

FIG. 8

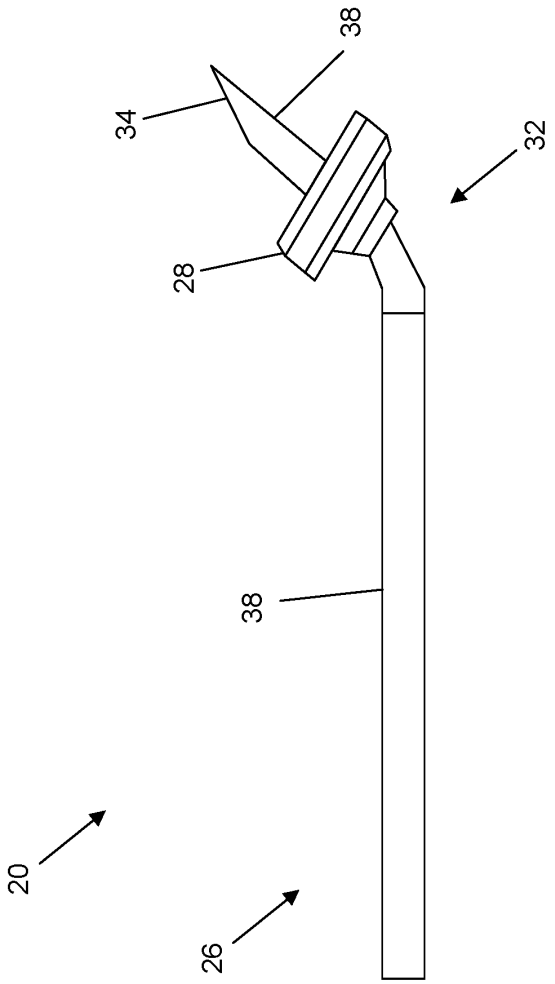

FIG. 9

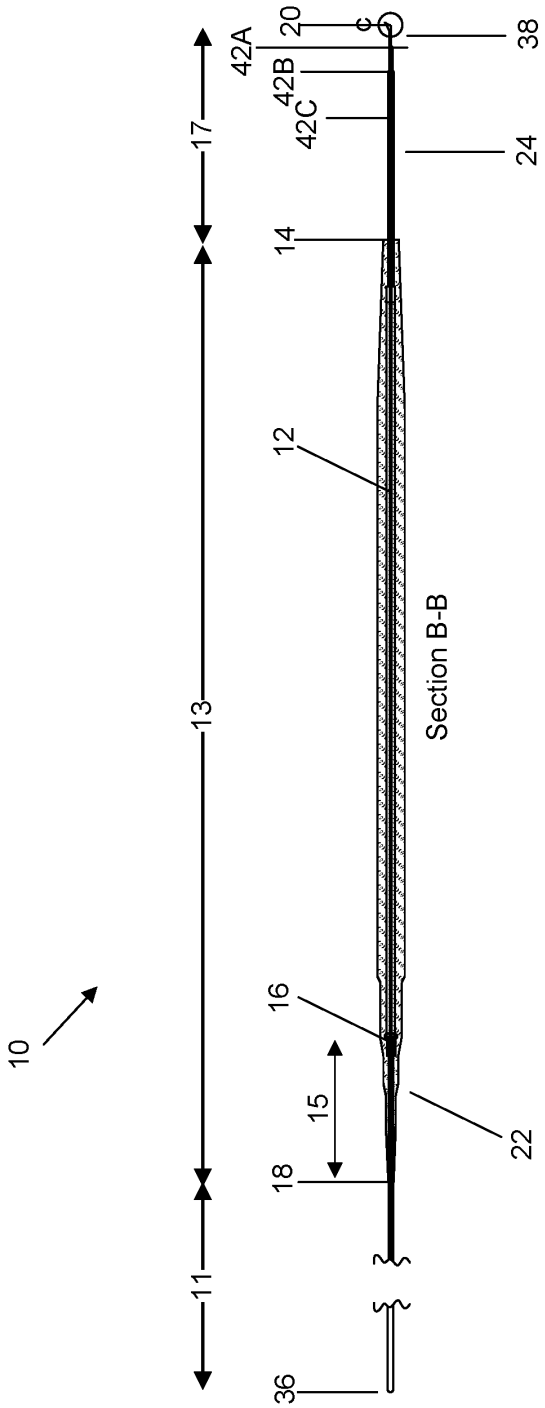

FIG. 10

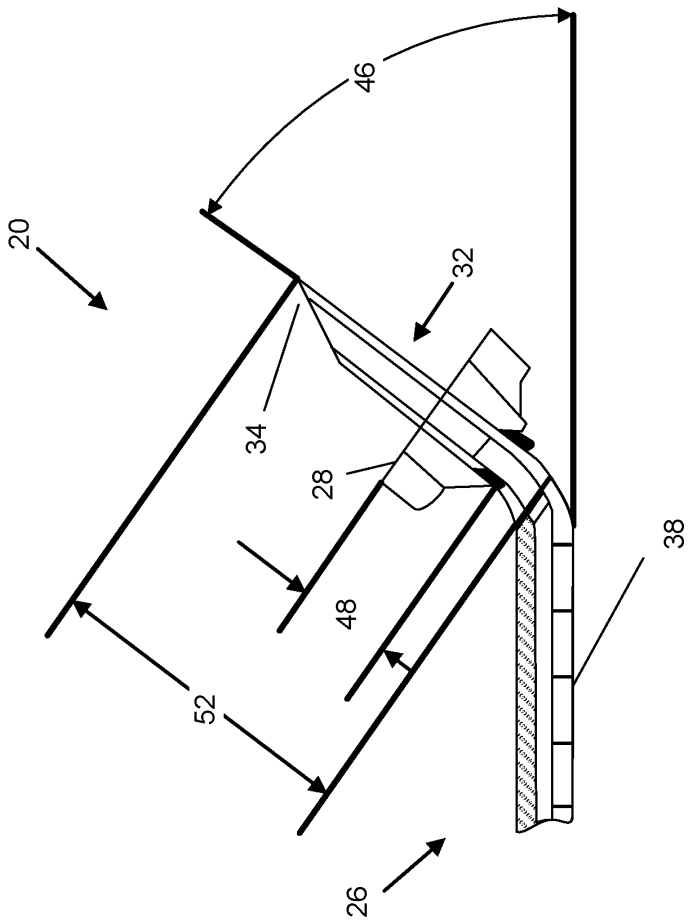

FIG. 11

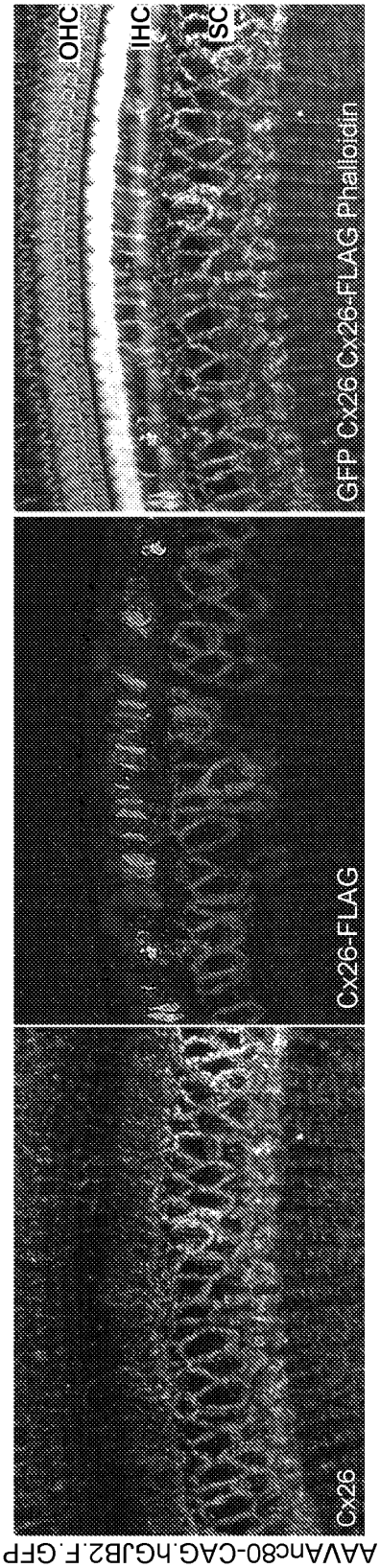
OHC
IHC
SC
GFP Cx26 Cx26-FLAG Phalloidin
Cx26-FLAG
Cx26
AAVAnc80-CAG.hGJB2.F.GFP

FIG. 12