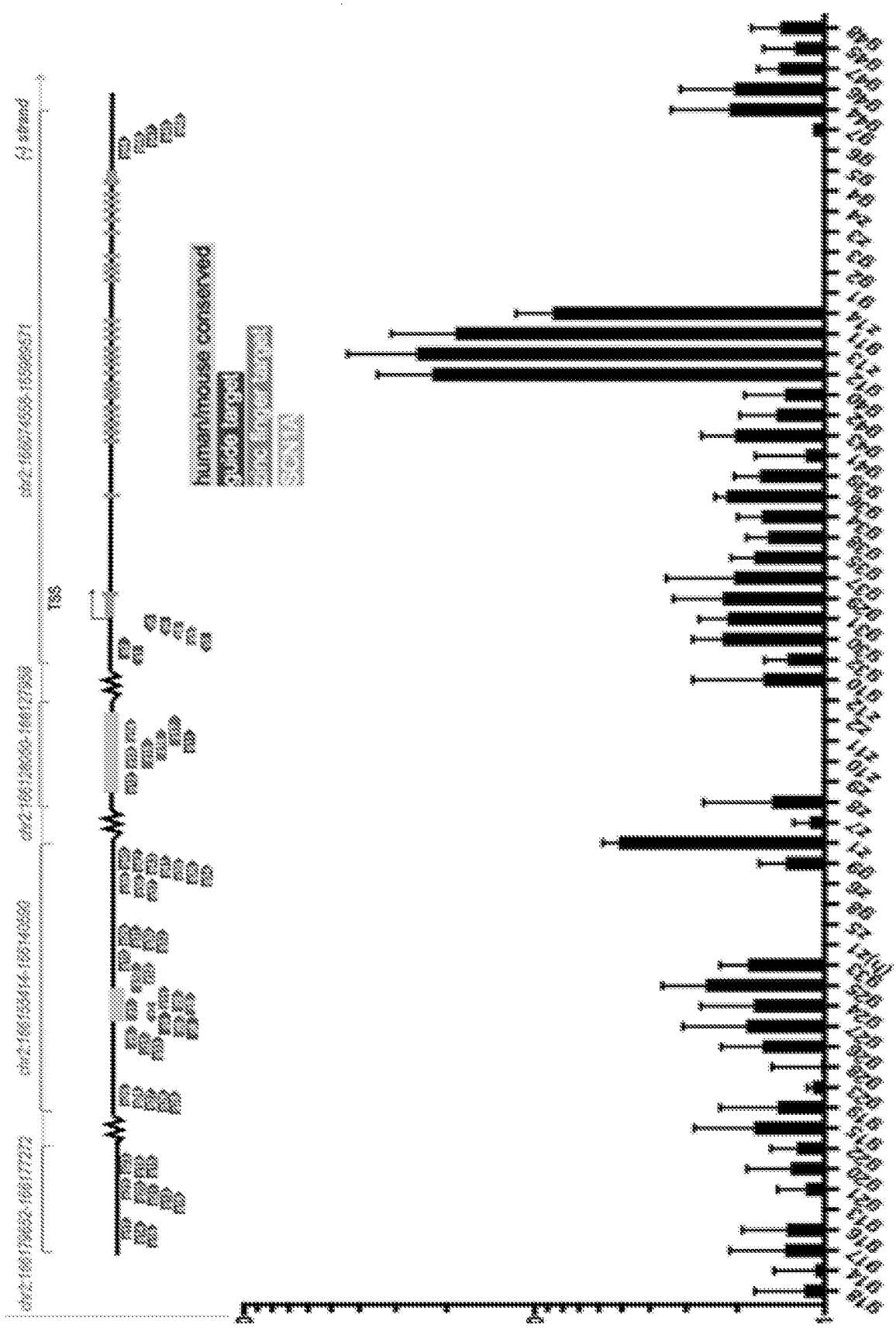


FIG. 1

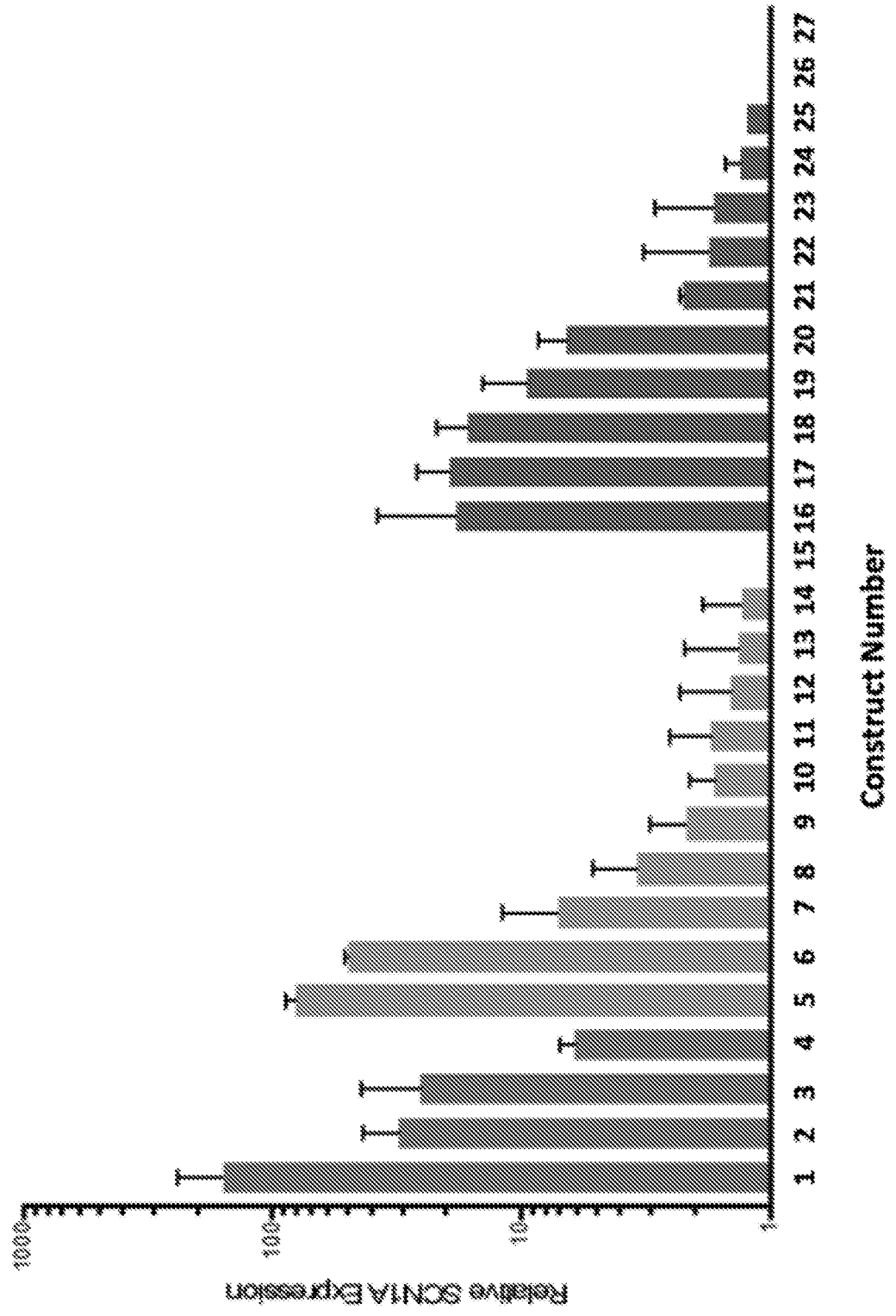

Relative SCN1A Expression
1000
100
10
1
1 2 3 4 5 6 7 8 9 10 11 12 13 14 15 16 17 18 19 20 21 22 23 24 25 26 27
Construct Number

FIG. 2A

FIG. 2B

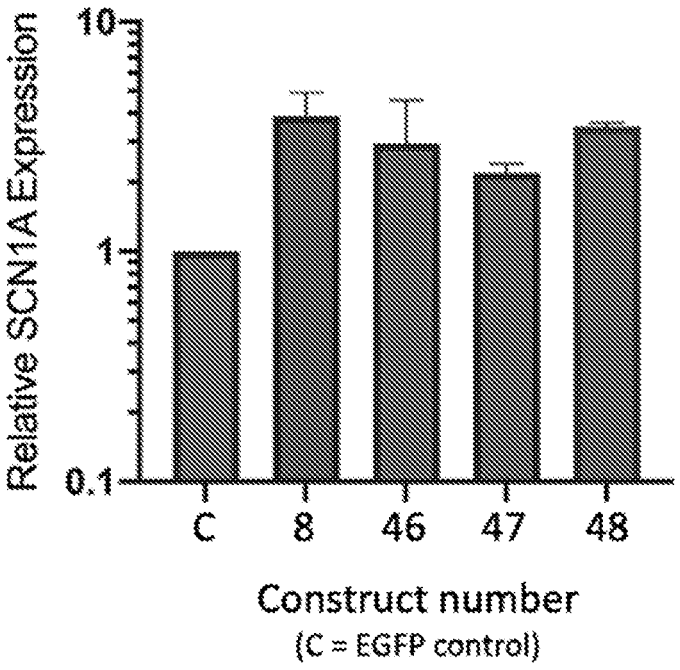

Relative SCN1A Expression
10
1
0.1
C
8
46
47
48
Construct number
(C = EGFP control)

FIG. 2C

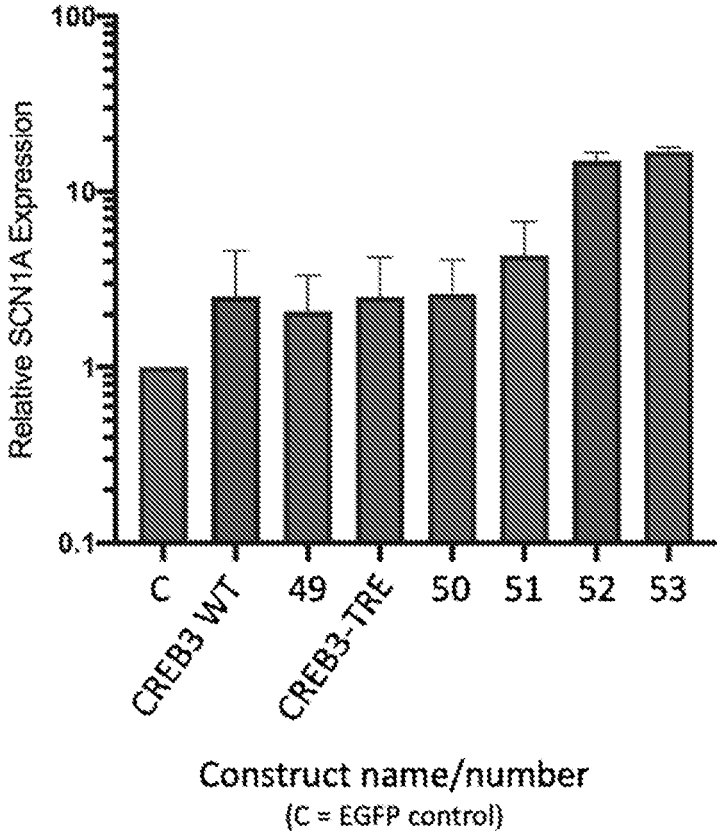

Relative SCN1A Expression
100
10
1
0.1
C
CREB3 WT
49
CREB3-TRE
50
51
52
53
Construct name/number
(C = EGFP control)

FIG. 3A

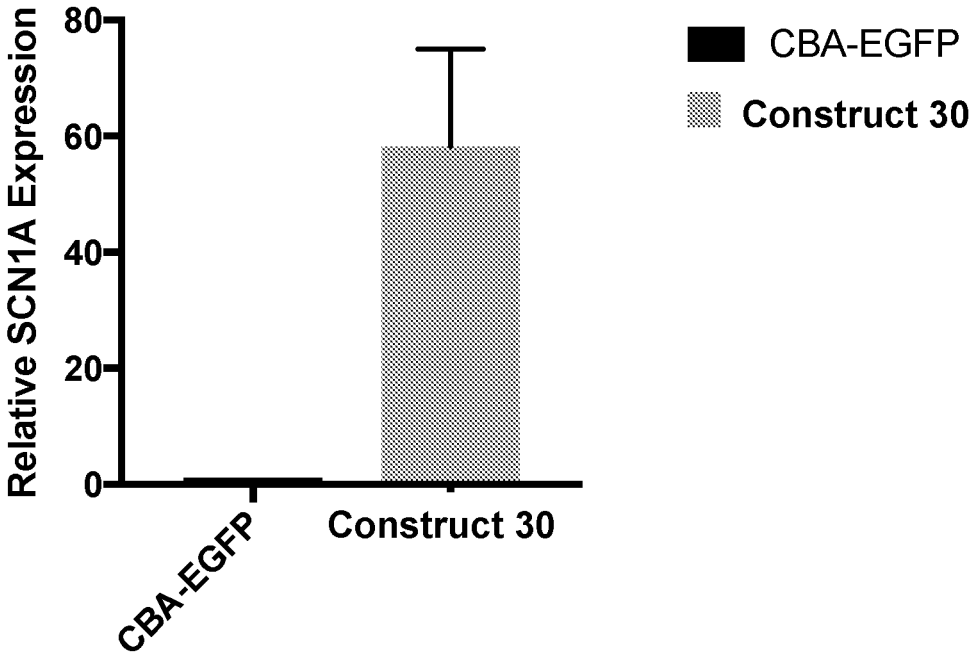
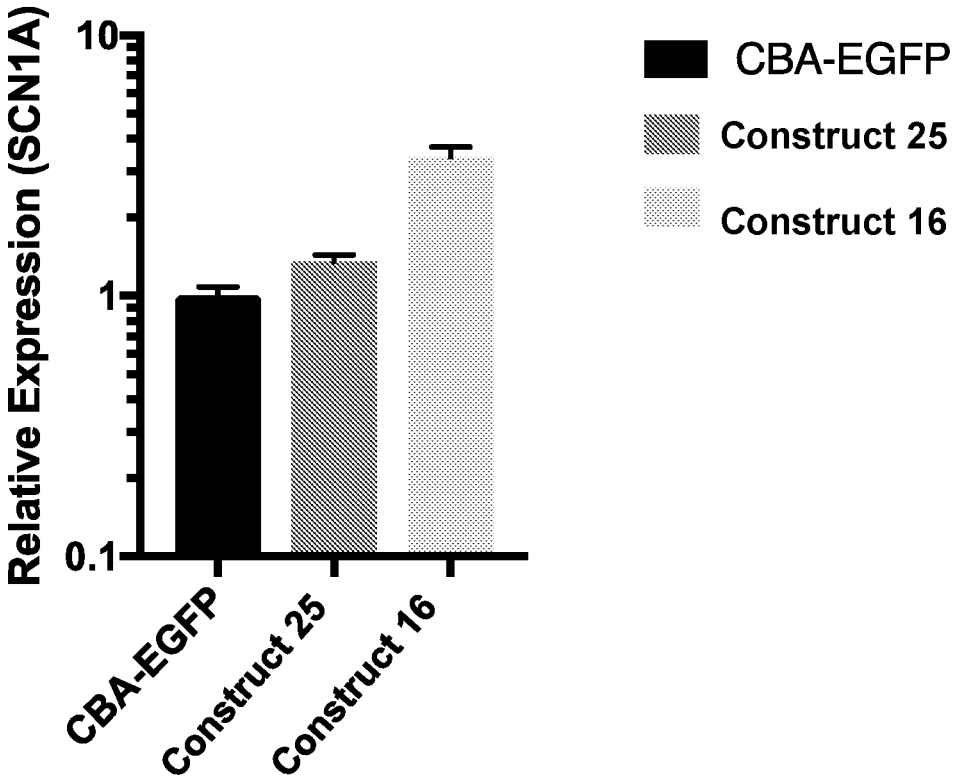

Relative SCN1A Expression
80
60
40
20
0
CBA-EGFP
Construct 30
CBA-EGFP
Construct 30

FIG. 3B


Relative Expression (SCN1A)
10
1
0.1
CBA-EGFP
Construct 25
Construct 16
CBA-EGFP
Construct 25
Construct 16

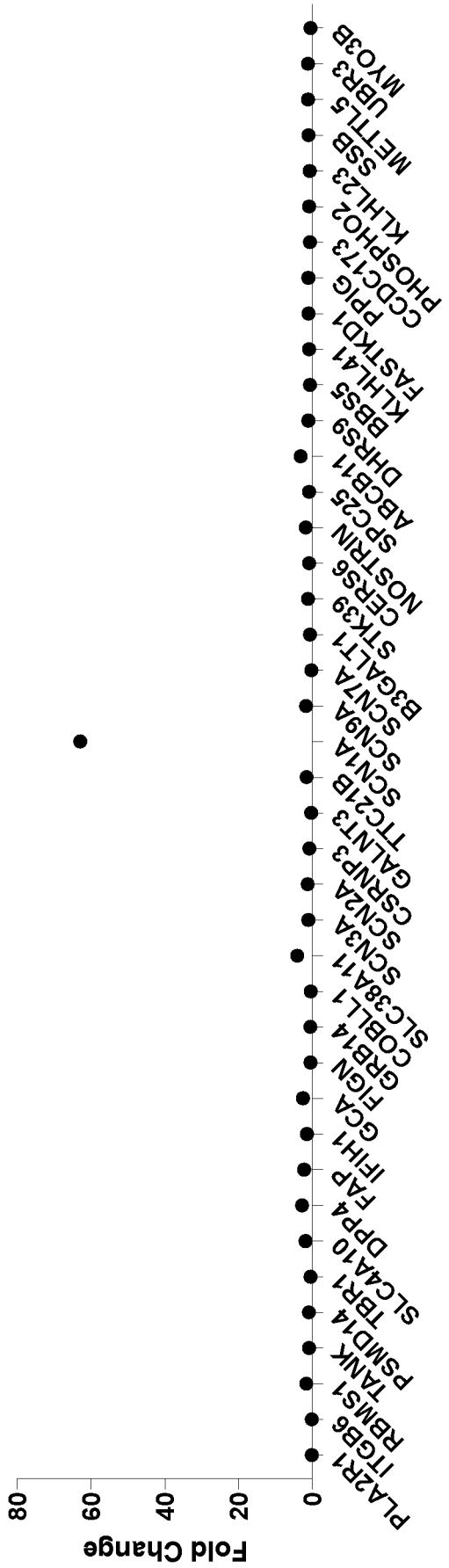

Fold Change
0
20
40
60
80
PLA2R1
ITGB6
RBMS1
TANK
PSMD14
TBR1
SLC4A10
DPP4
FAP
IFIH1
GCA
FIGN
GRB14
COBLL1
SLC38A11
SCN3A
CSRNP3
GALNT3
TTC21B
SCN1A
SCN9A
B3GALT1
STK39
CERS6
NOSTRIN
SPC25
ABCB11
DHRS9
BBS5
KLHL41
FASTKD1
PPIG
CCDC173
PHOSPHO2
KLHL23
SSB
METTL5
UBR3
MYO3B

FIG. 4

FIG. 5A

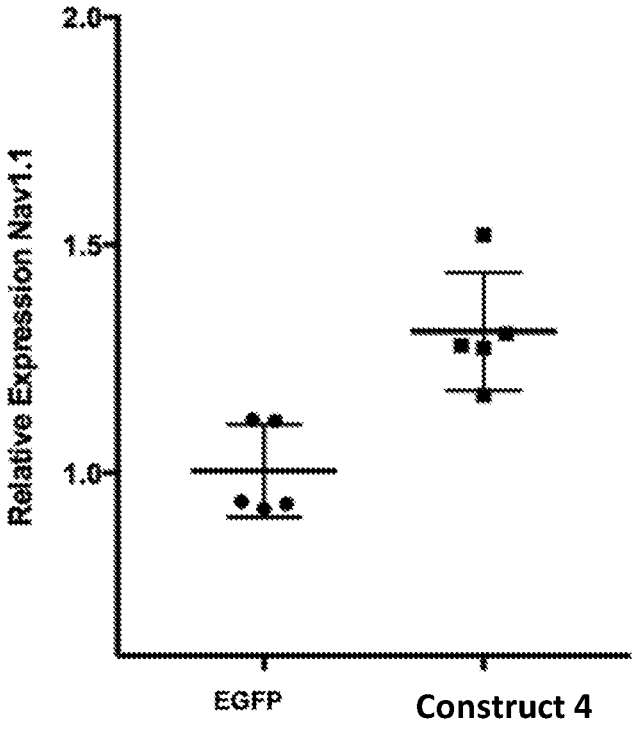

Relative Expression Nav1.1
2.0
1.5
1.0
EGFP
Construct 4

FIG. 5B

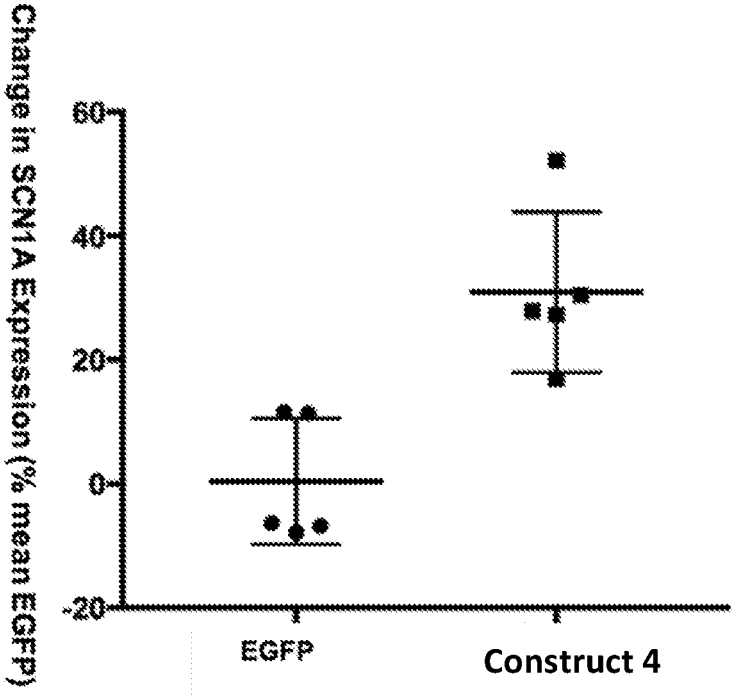

Change in SCN1A Expression (% mean EGFP)
60
40
20
0
-20
EGFP
Construct 4

FIG. 6A

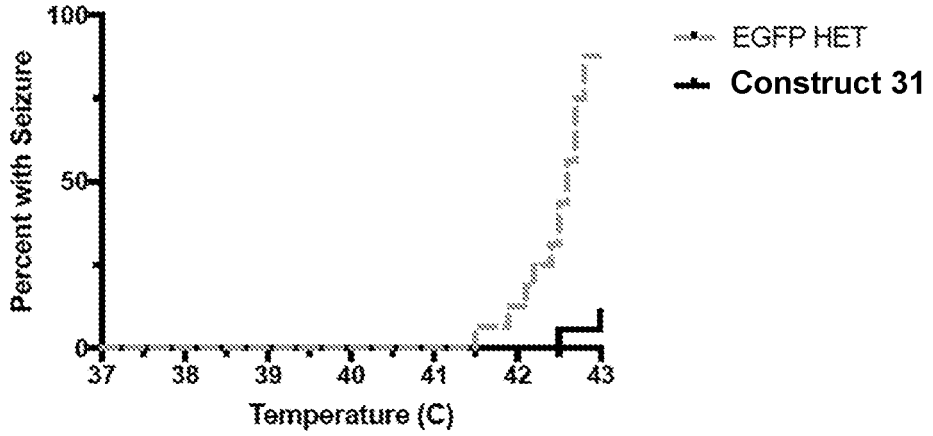

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP HET
Construct 31

FIG. 6B

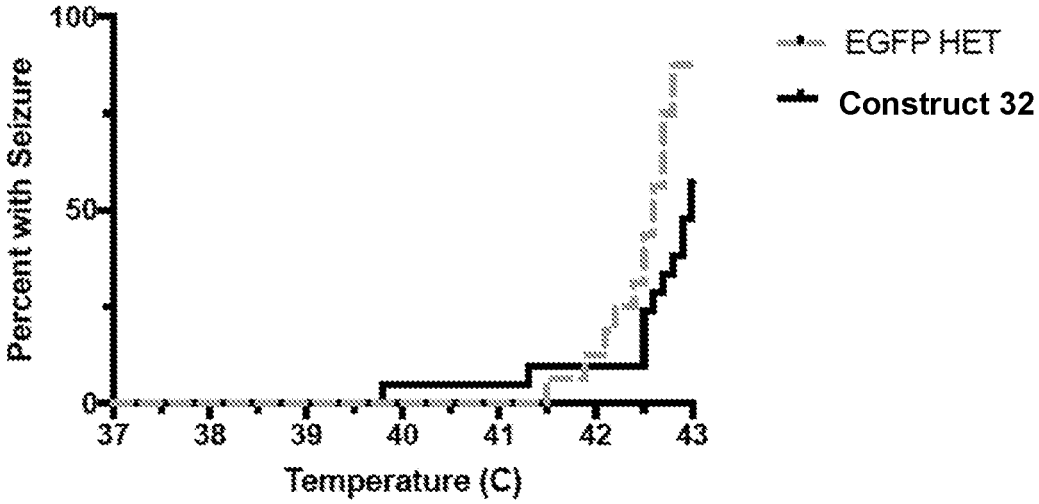

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP HET
Construct 32

FIG. 6C

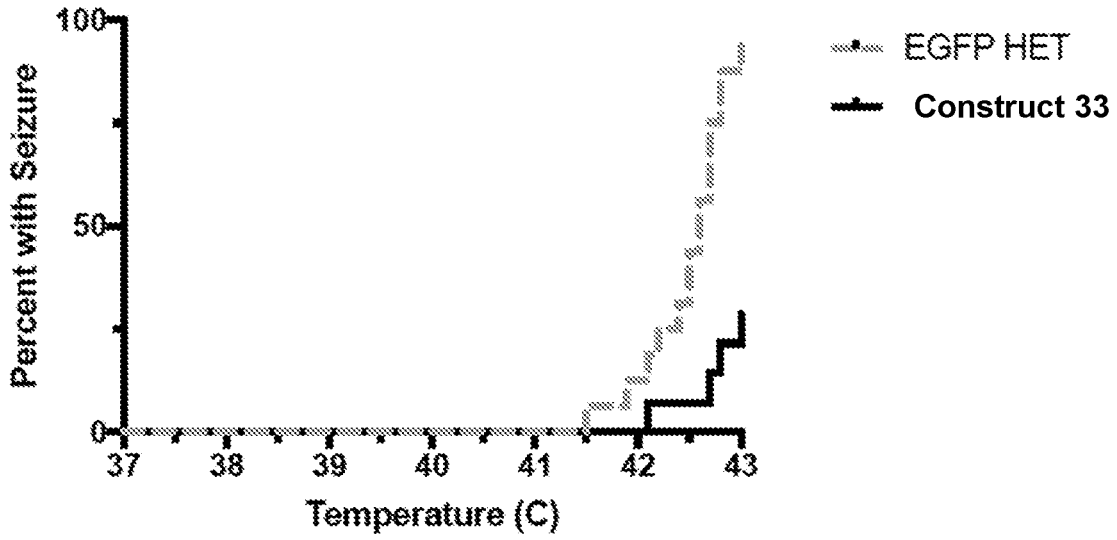

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP HET
Construct 33

FIG. 6D

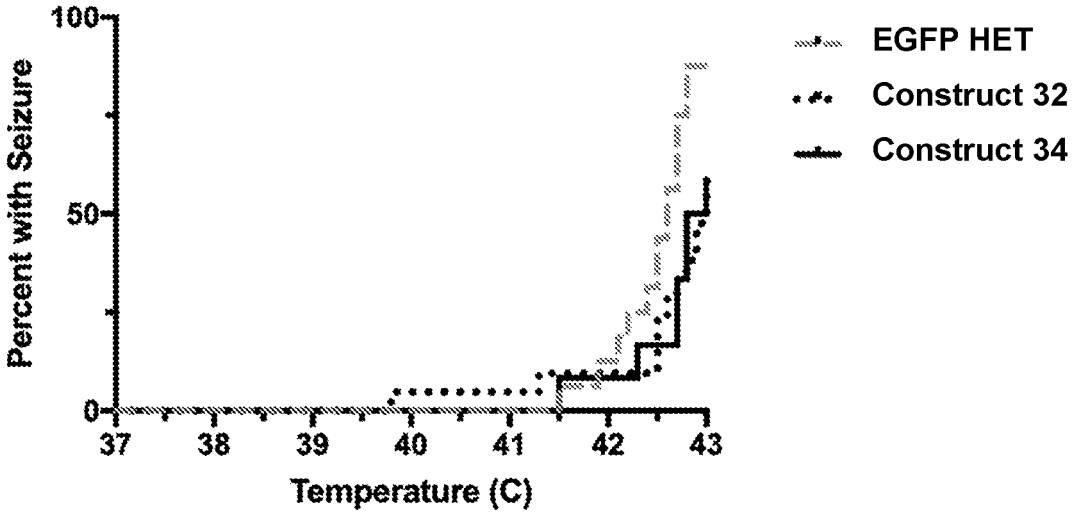

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP HET
Construct 32
Construct 34

FIG. 6E

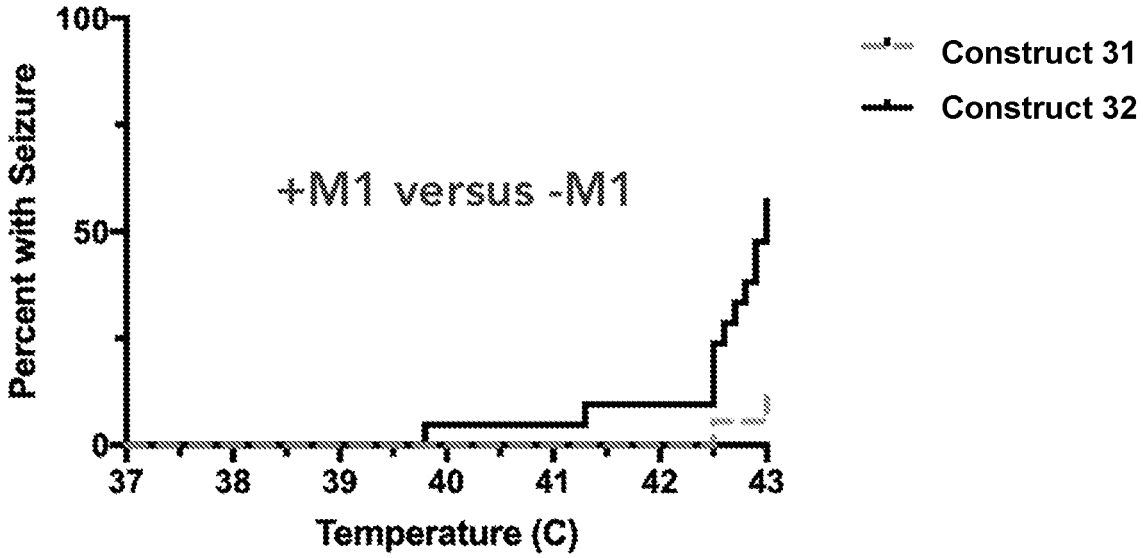

100
50
0
Percent with Seizure
+M1 versus -M1
37
38
39
40
41
42
43
Temperature (C)
Construct 31
Construct 32

FIG. 6F

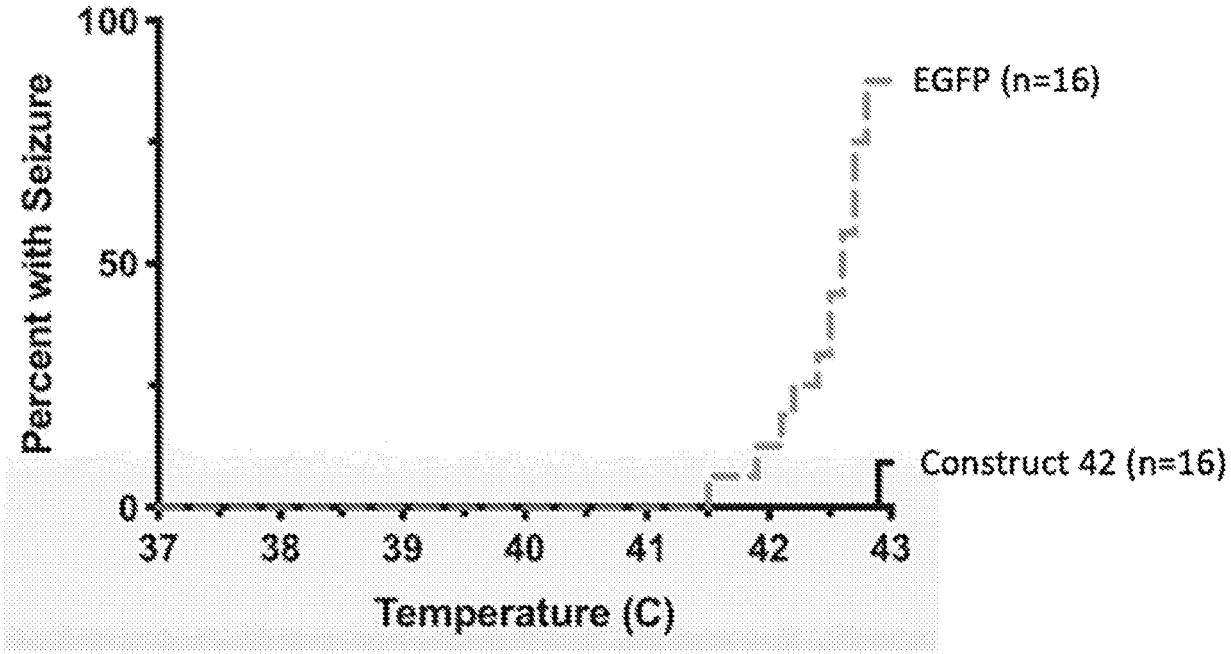

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP (n=16)
Construct 42 (n=16)

FIG. 6G

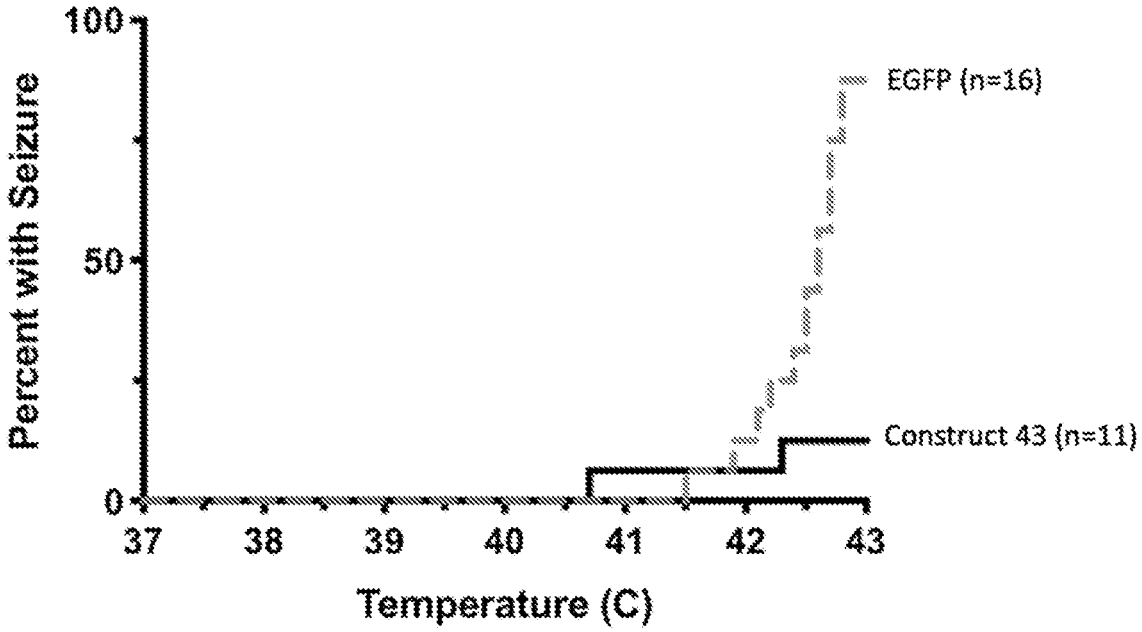

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
EGFP (n=16)
Construct 43 (n=11)

FIG. 6H

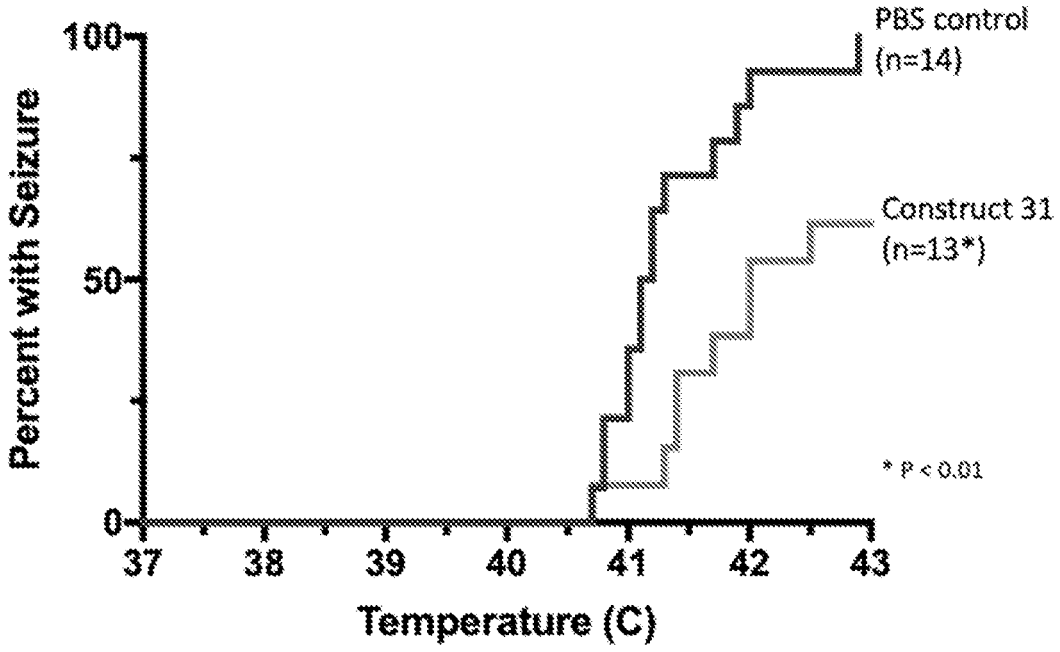

100
50
0
Percent with Seizure
37
38
39
40
41
42
43
Temperature (C)
PBS control
(n=14)
Construct 31
(n=13*)
* P < 0.01

FIG. 7A

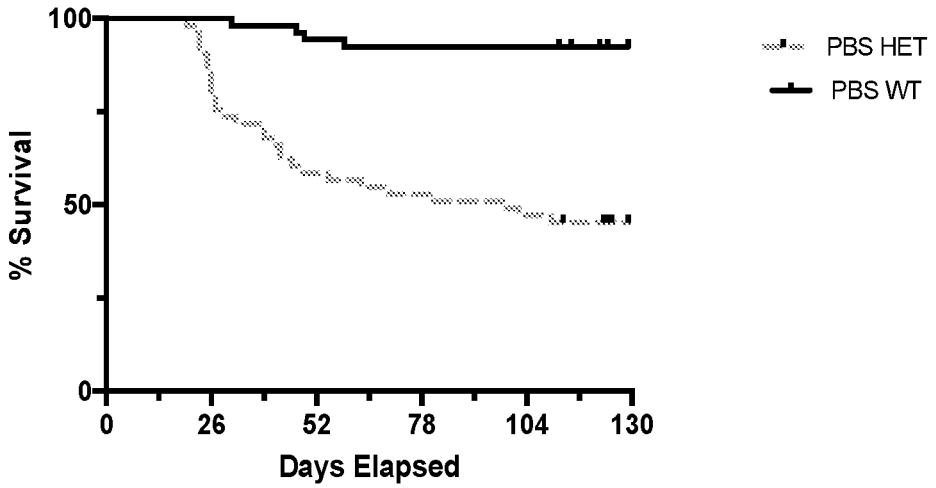

100
50
0
% Survival
0
26
52
78
104
130
Days Elapsed
PBS HET
PBS WT

FIG. 7B

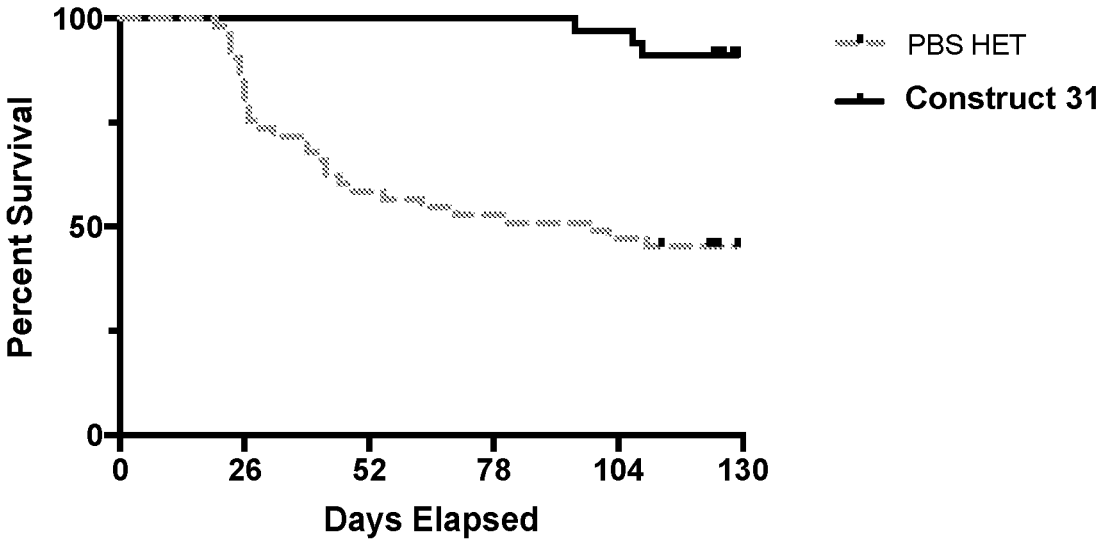

100
50
0
Percent Survival
0
26
52
78
104
130
Days Elapsed
PBS HET
Construct 31

FIG. 7C

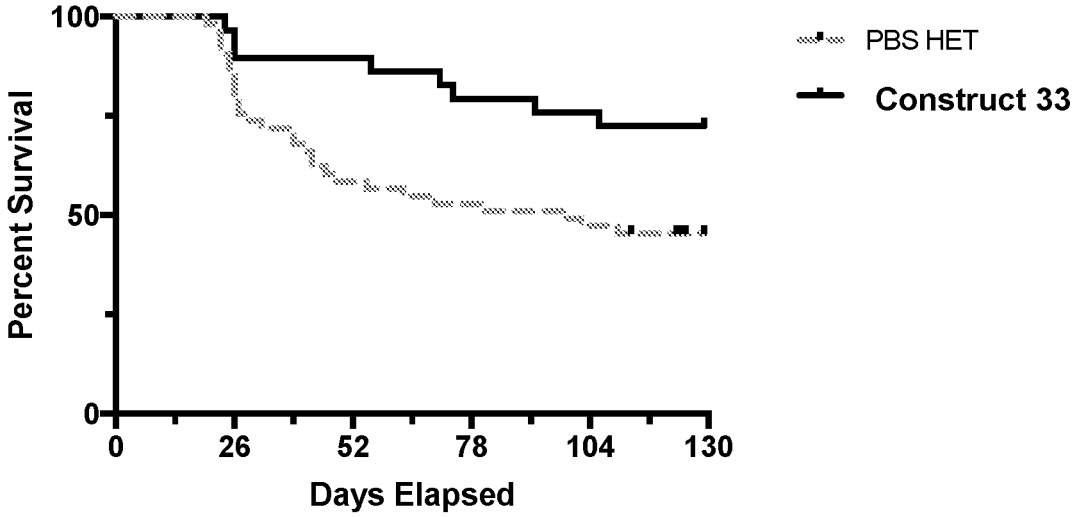

100
50
0
Percent Survival
0
26
52
78
104
130
Days Elapsed
PBS HET
Construct 33

FIG. 7D

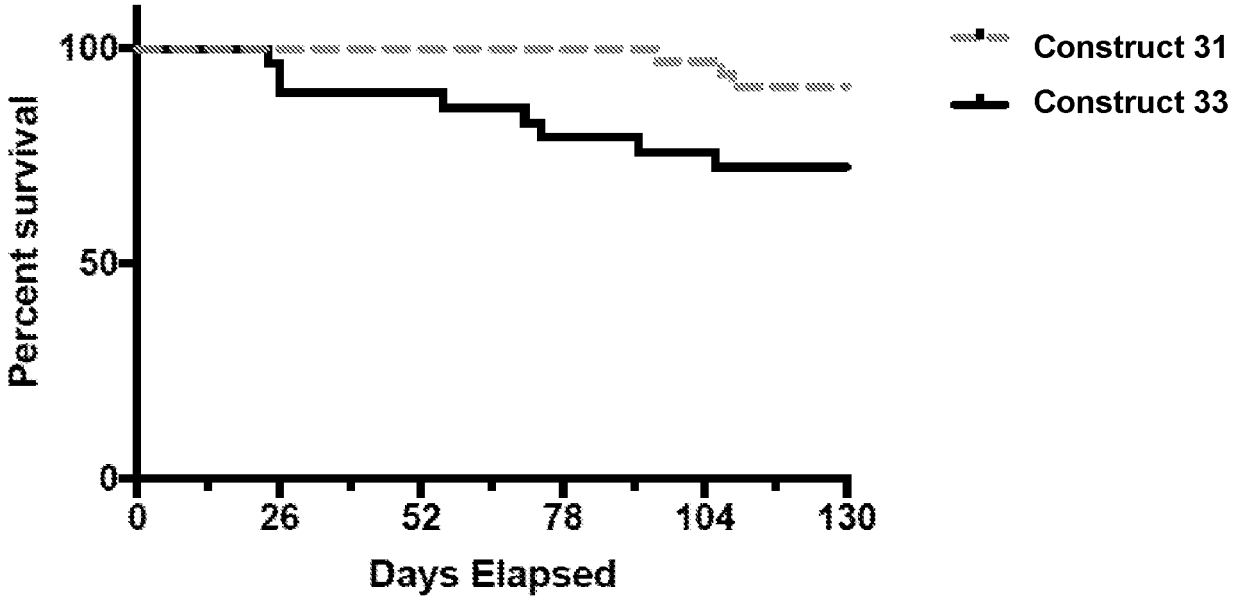

100
50
0
Percent survival
0
26
52
78
104
130
Days Elapsed
Construct 31
Construct 33

FIG. 7E

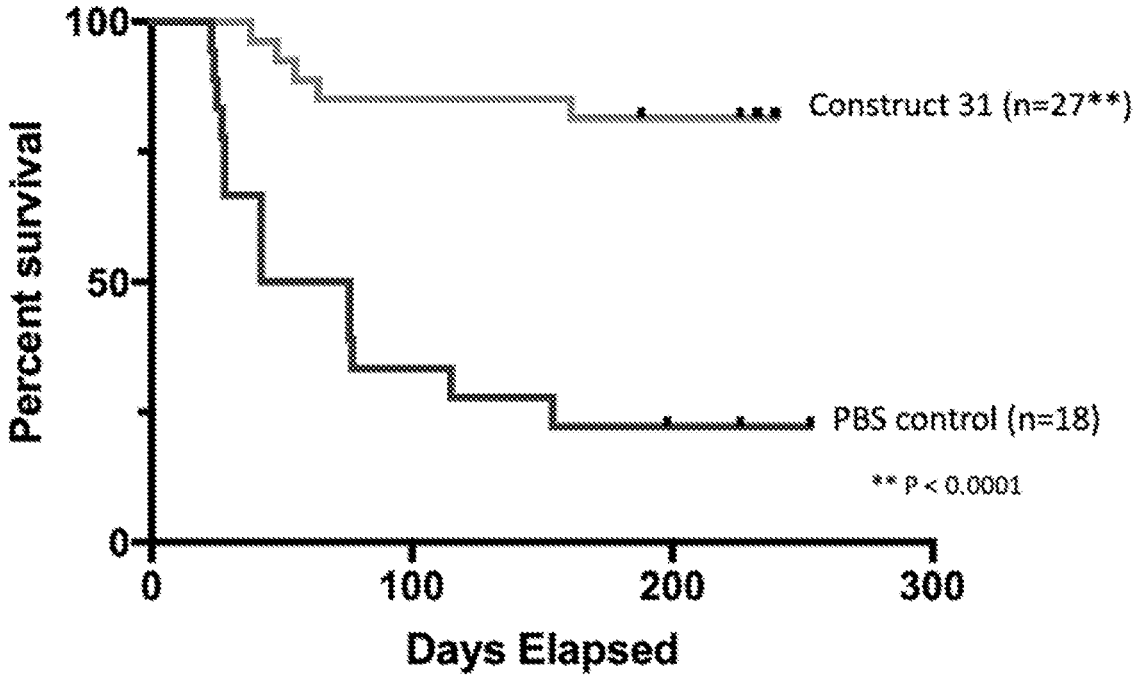

100
50
0
Percent survival
0
100
200
300
Days Elapsed
Construct 31 (n=27**)
PBS control (n=18)
** P < 0.0001

FIG. 8

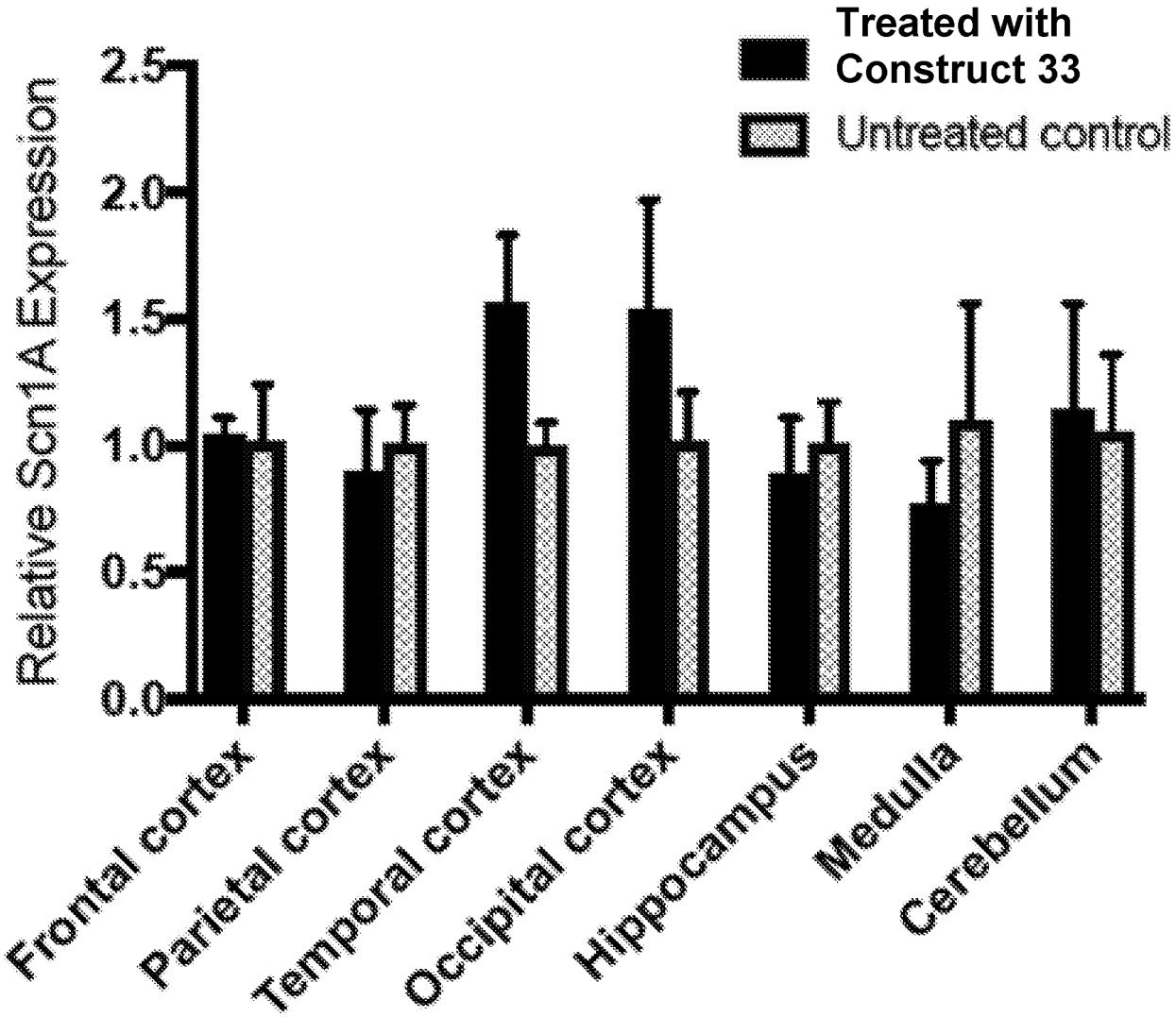

Treated with Construct 33
Untreated control
Relative Scn1A Expression
2.5
2.0
1.5
1.0
0.5
0.0
Frontal cortex
Parietal cortex
Temporal cortex
Occipital cortex
Hippocampus
Medulla
Cerebellum

FIG. 9

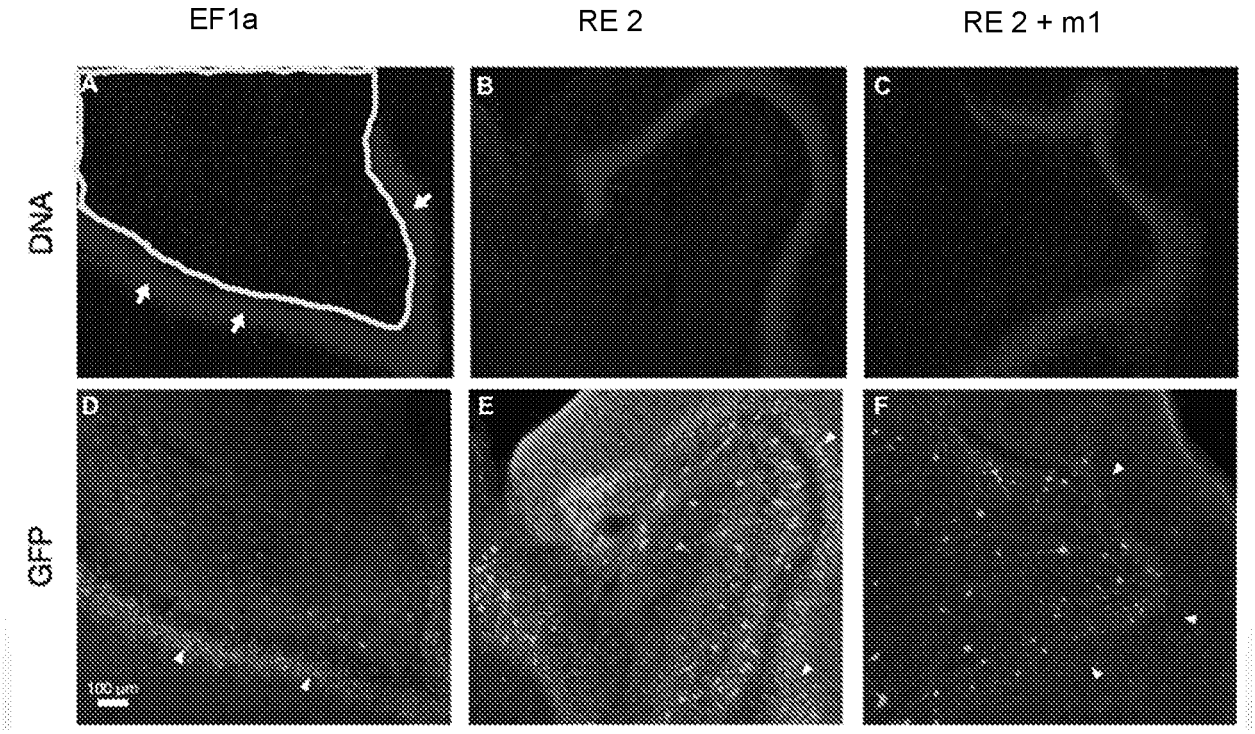
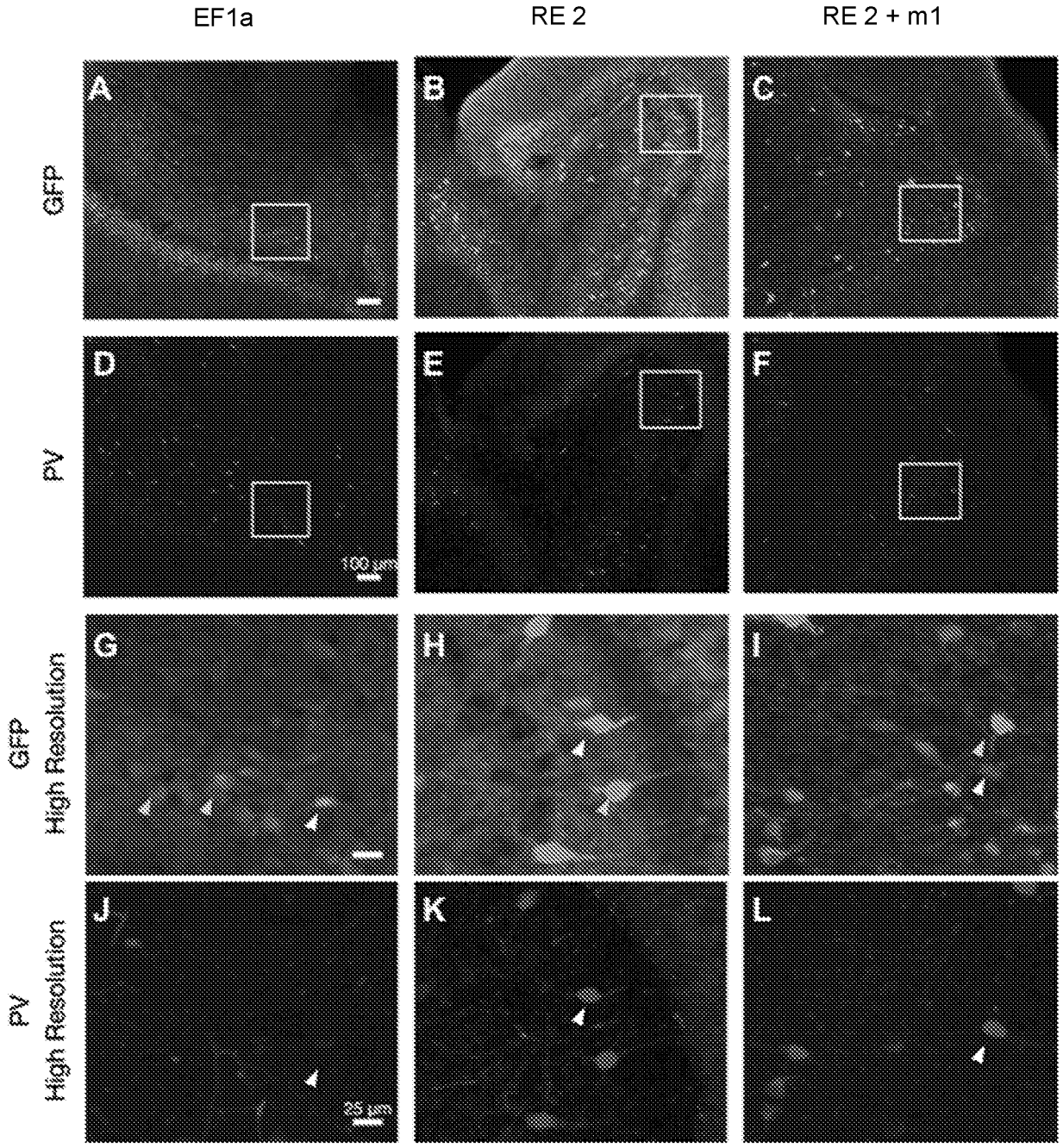


FIG. 10


EF1a
RE 2
RE 2 + m1
GFP
PV
GFP
High Resolution
PV
High Resolution
A
B
C
D
E
F
G
H
I
J
K
L
100 μm
25 μm

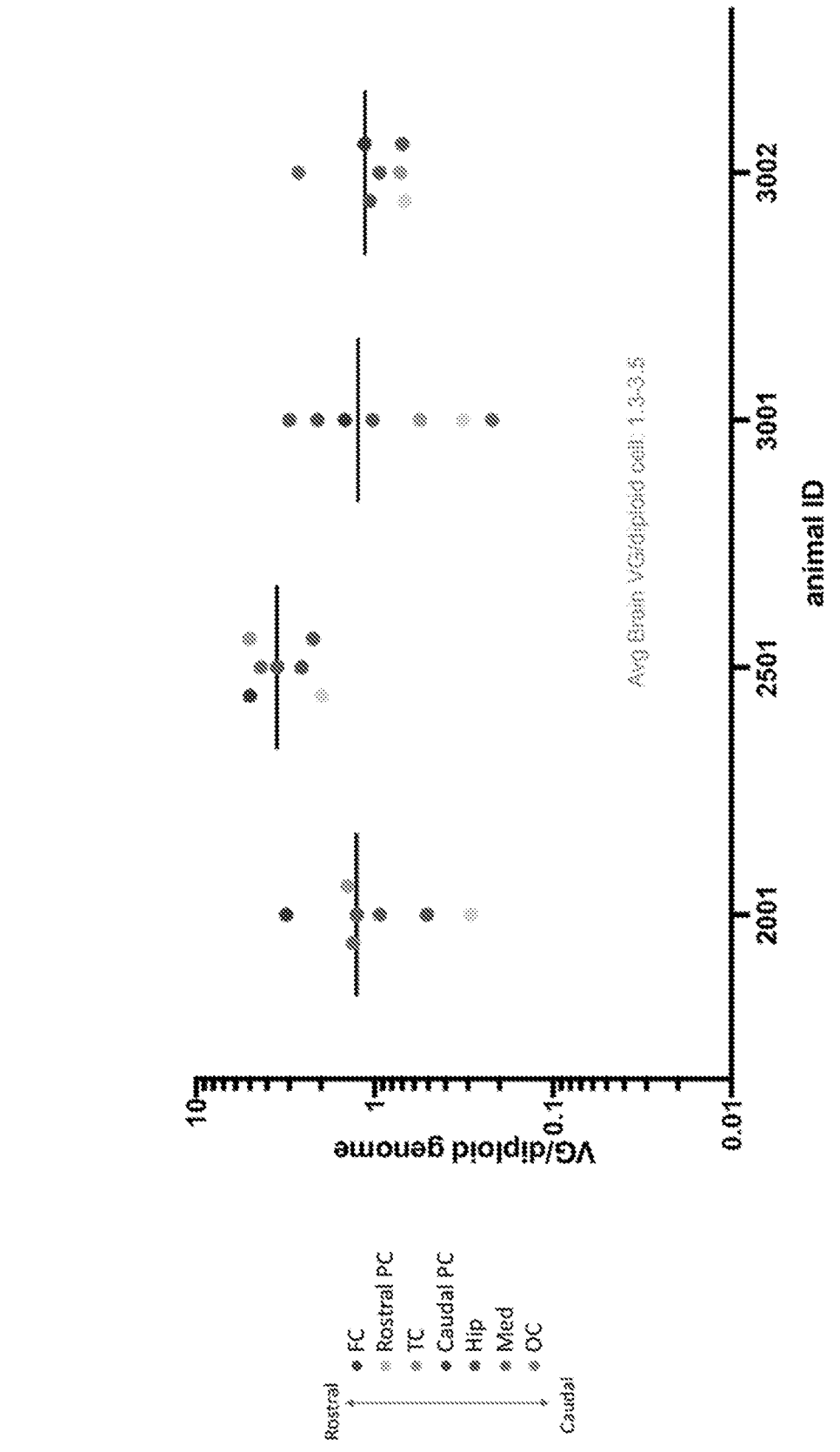

Avg Brain VG/diploid cell 1.3-3.5
VG/diploid genome
10
1
0.1
0.01
animal ID
2001
2501
3001
3002
Rostral
Caudal
FC
Rostral PC
TC
Caudal PC
Hip
Med
OC

FIG. 11

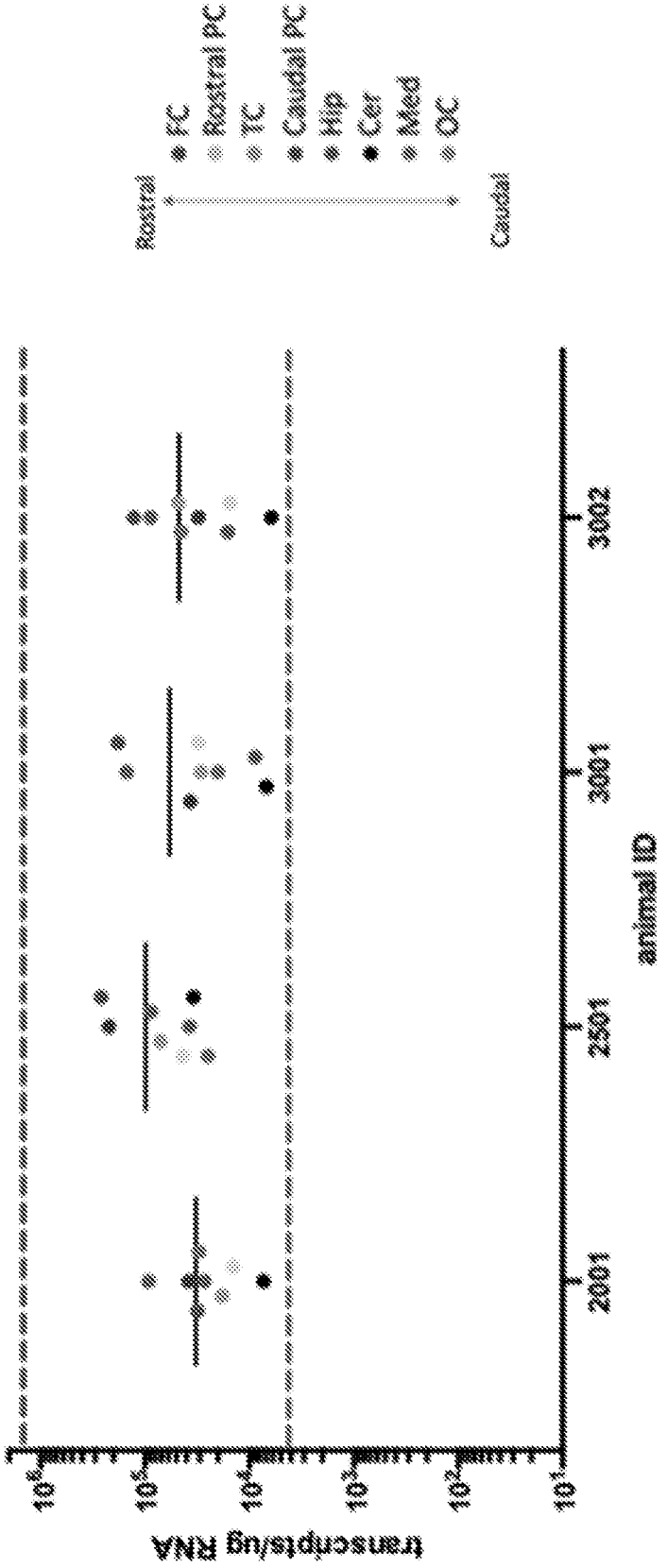

Rostral
Caudal
FC
Rostral PC
TC
Caudal PC
Hip
Cer
Med
OC
transcripts/ug RNA
animal ID
2001
2501
3001
3002

FIG. 12

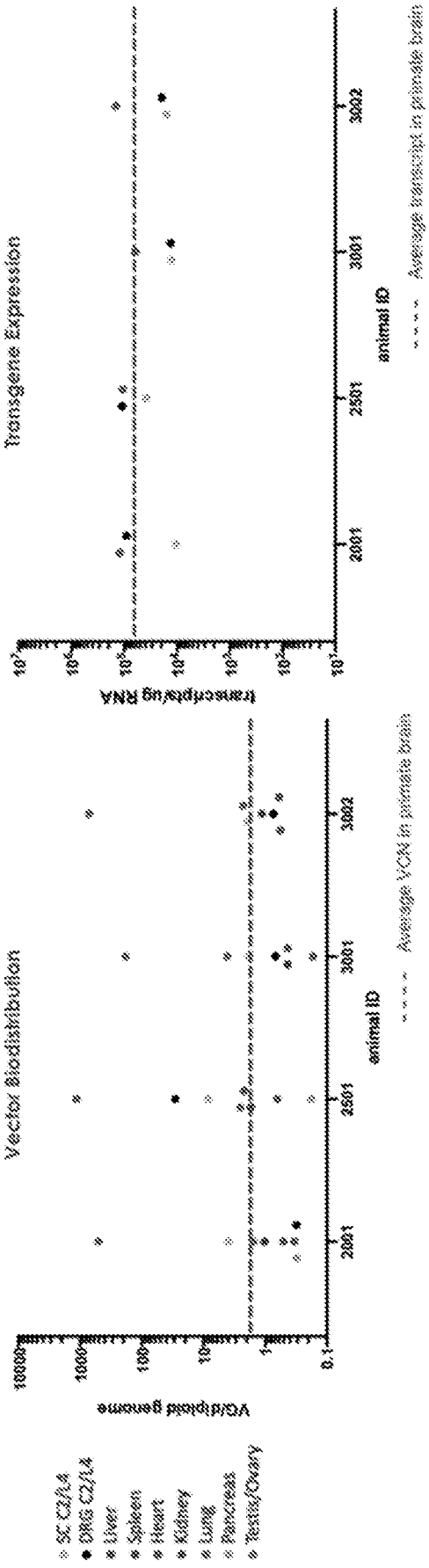

Transgene Expression
transcripts/ug RNA
animal ID
Average transcript in primate brain
Vector Biodistribution
VG/diploid genome
animal ID
Average VGN in primate brain
SC C2/L4
DRG C2/L4
Liver
Spleen
Heart
Kidney
Lung
Pancreas
Testis/Ovary

FIG. 13