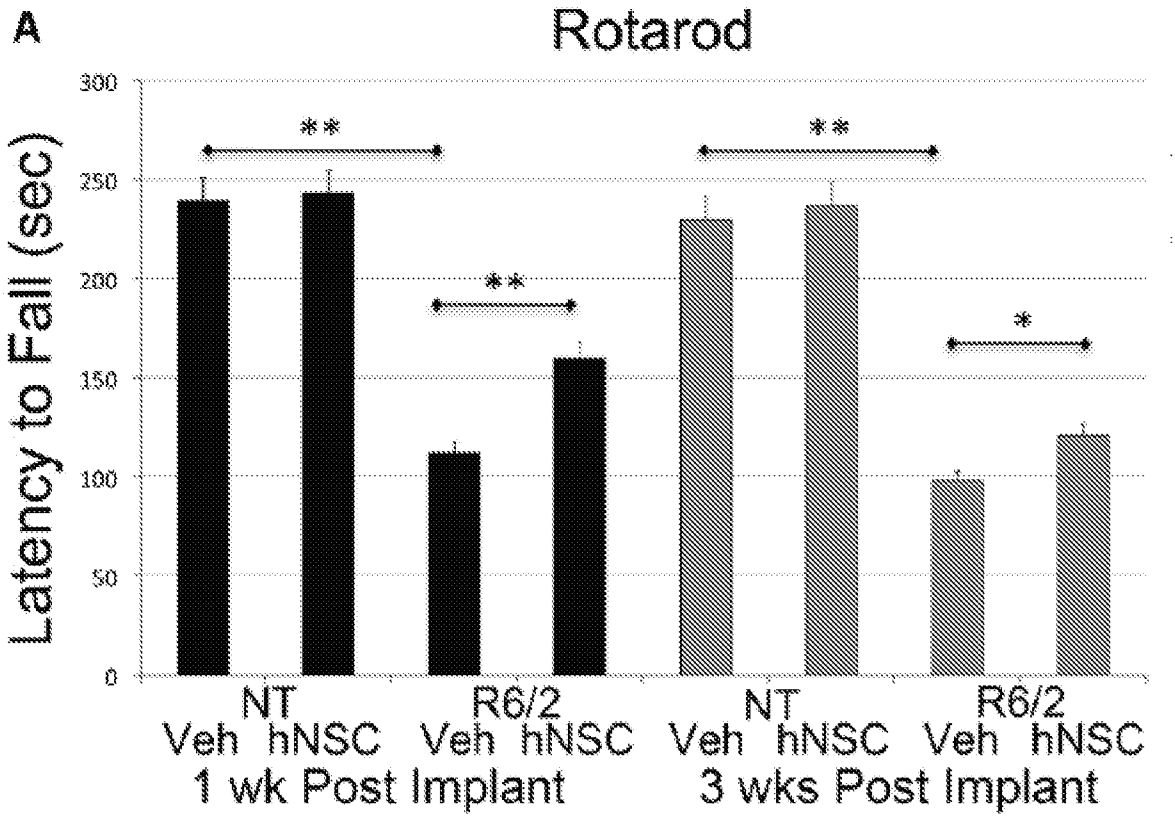

A
Rotarod
Latency to Fall (sec)
300
250
200
150
100
50
0
**
**
**
*
NT
R6/2
NT
R6/2
Veh hNSC Veh hNSC Veh hNSC Veh hNSC
1 wk Post Implant
3 wks Post Implant

FIG. 1A

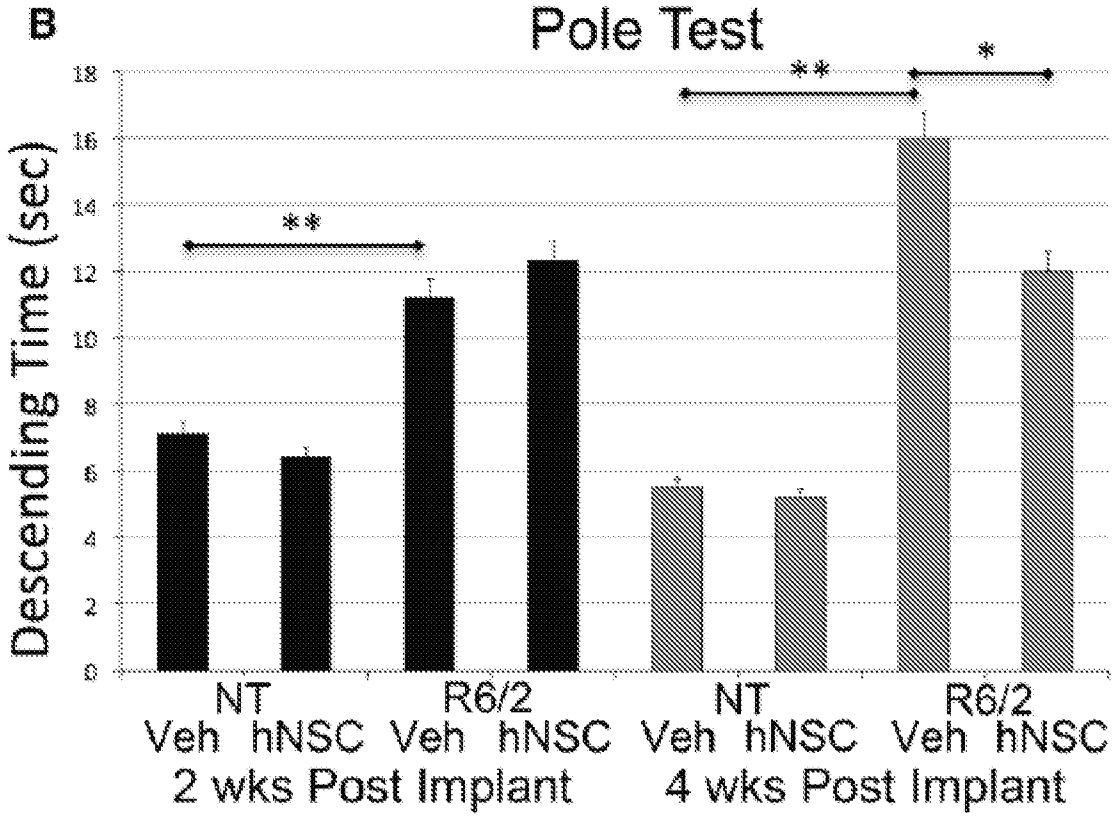

B
Pole Test
Descending Time (sec)
0
2
4
6
8
10
12
14
16
18
**
**
*
NT
R6/2
NT
R6/2
Veh hNSC Veh hNSC Veh hNSC Veh hNSC
2 wks Post Implant
4 wks Post Implant

FIG. 1B

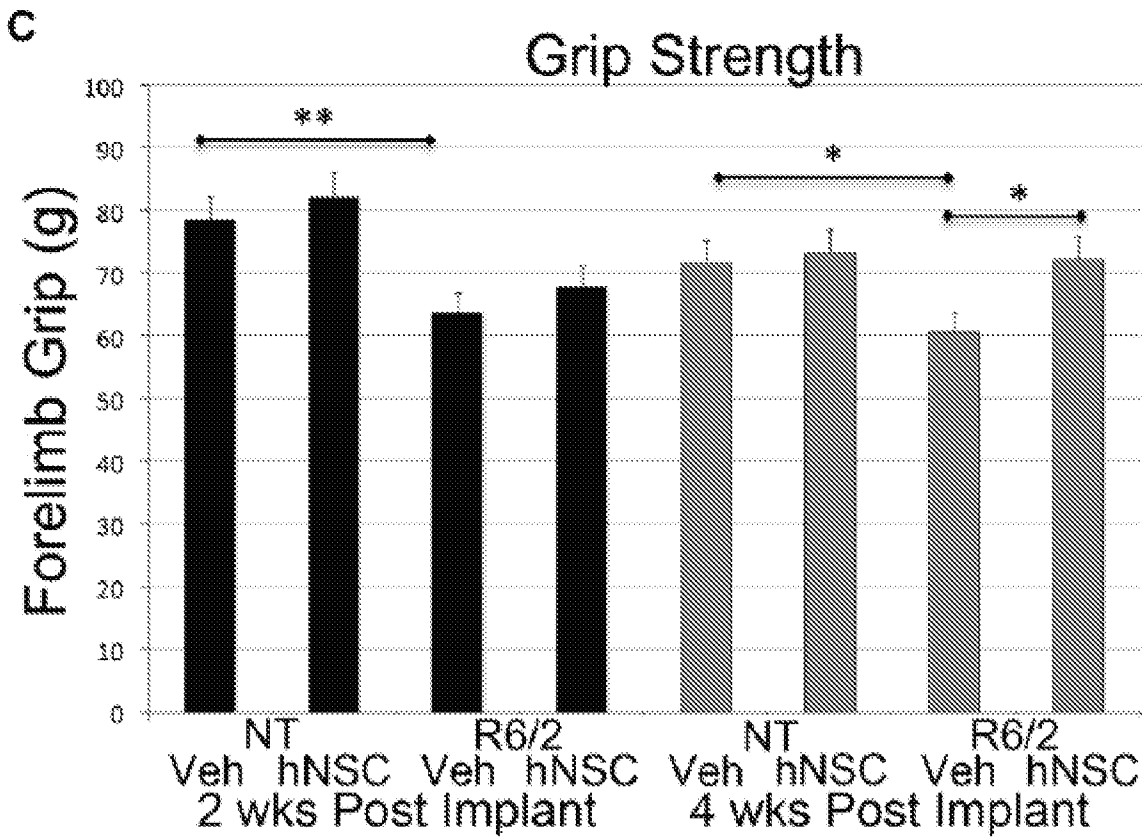

c
Grip Strength
Forelimb Grip (g)
100
90
80
70
60
50
40
30
20
10
0
**
*
*
NT
R6/2
NT
R6/2
Veh hNSC Veh hNSC Veh hNSC Veh hNSC
2 wks Post Implant
4 wks Post Implant

FIG. 1C

D

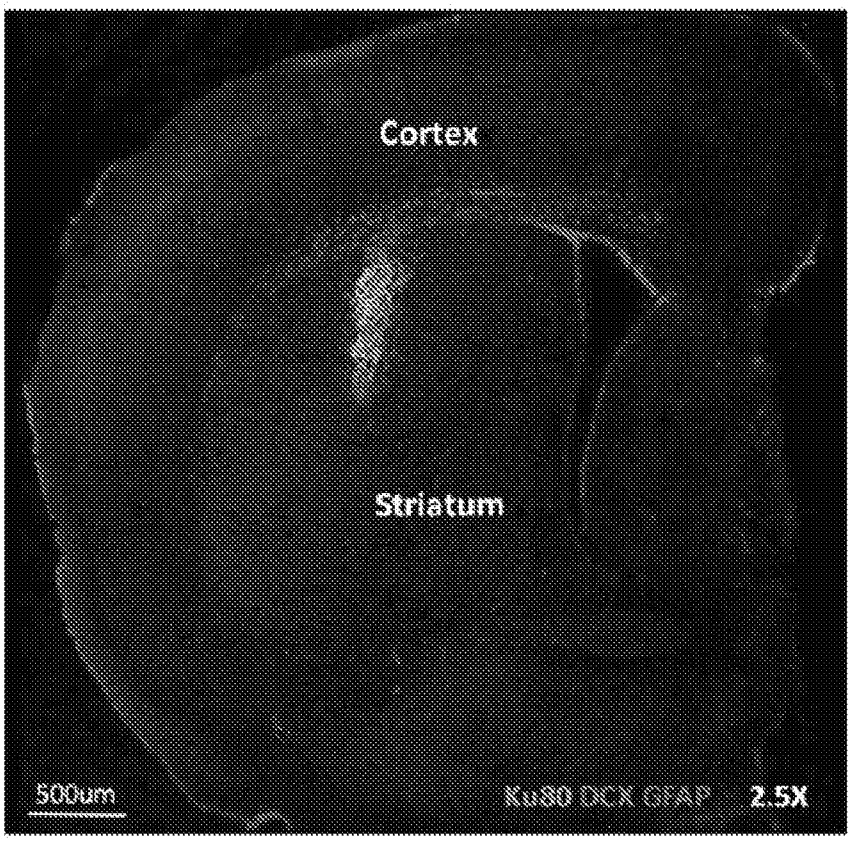
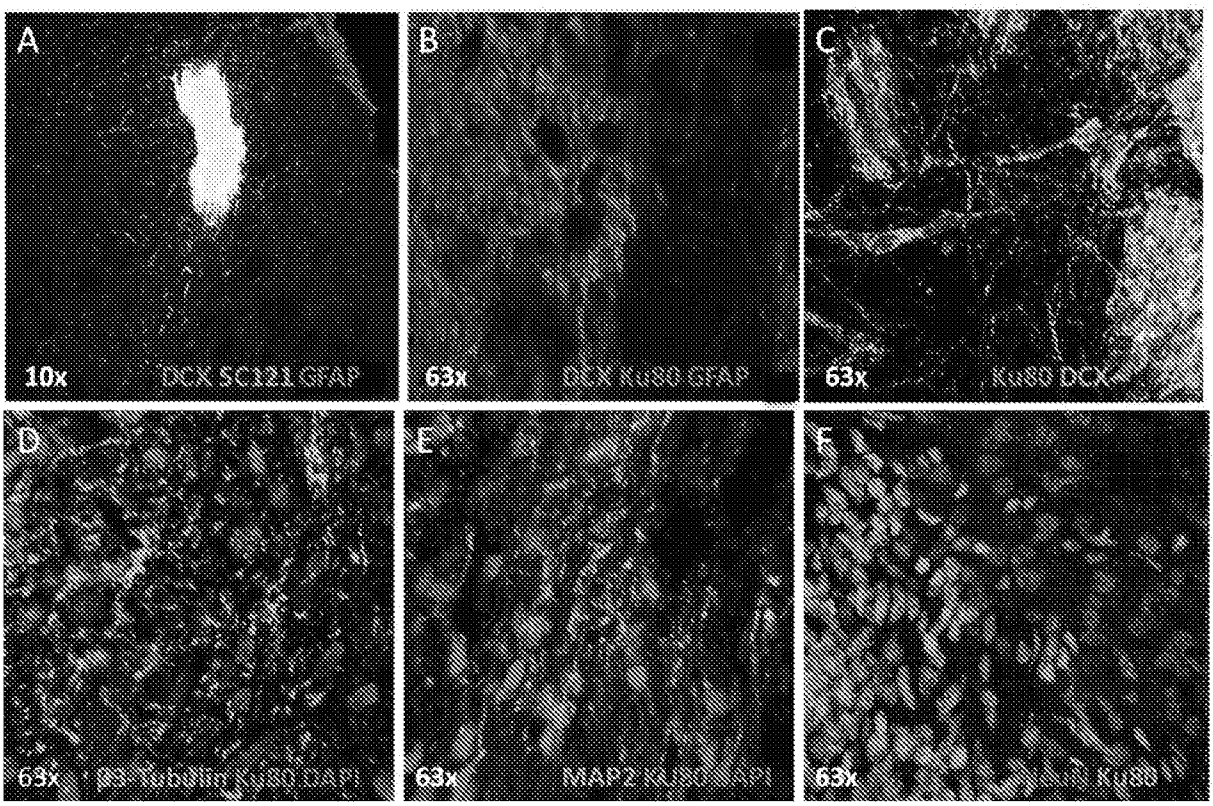
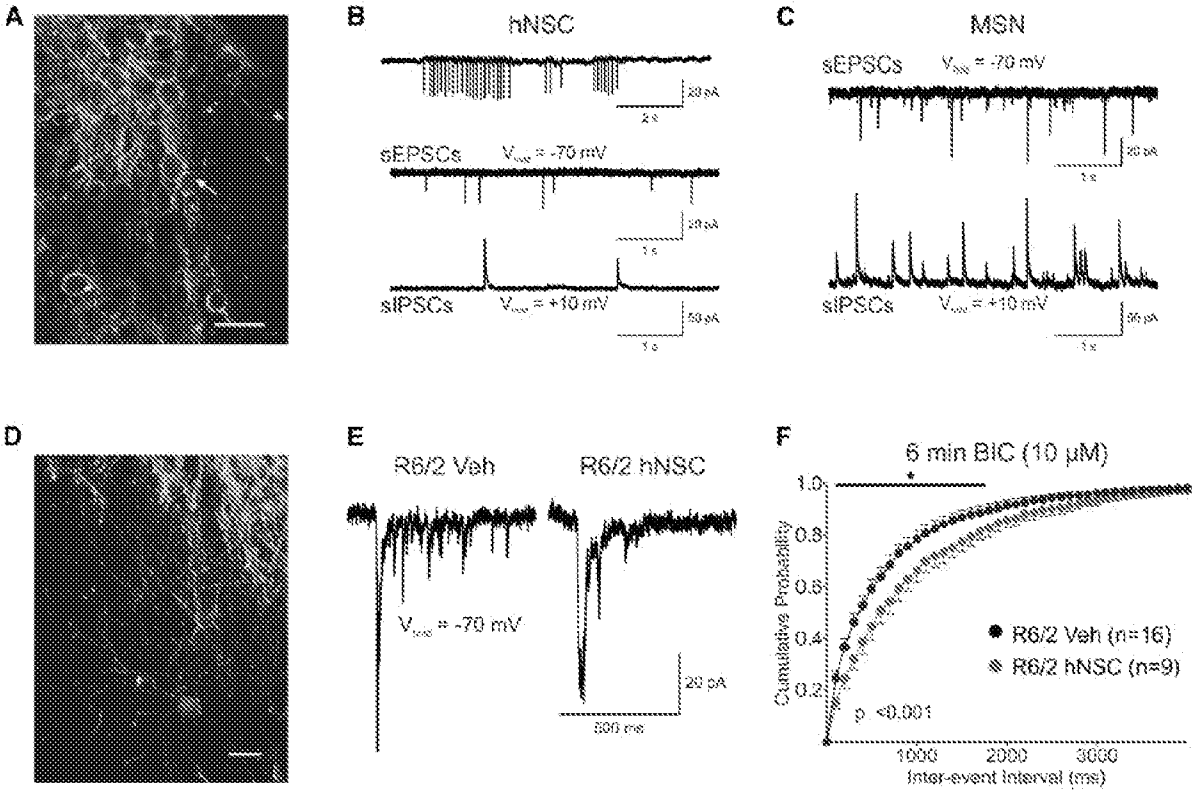


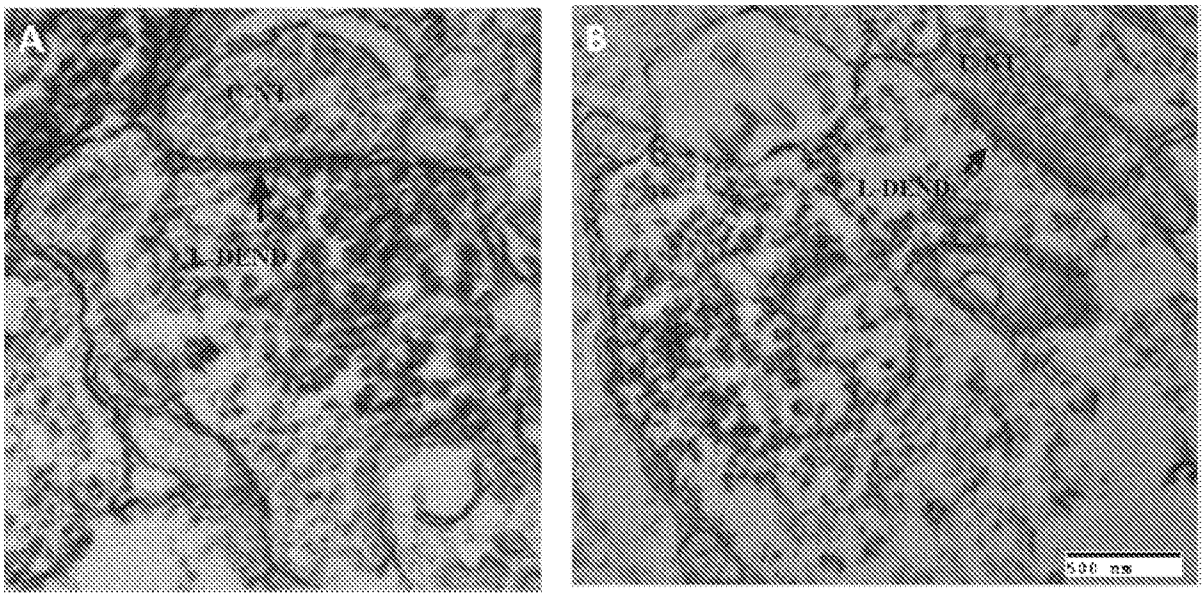
FIG. 1D



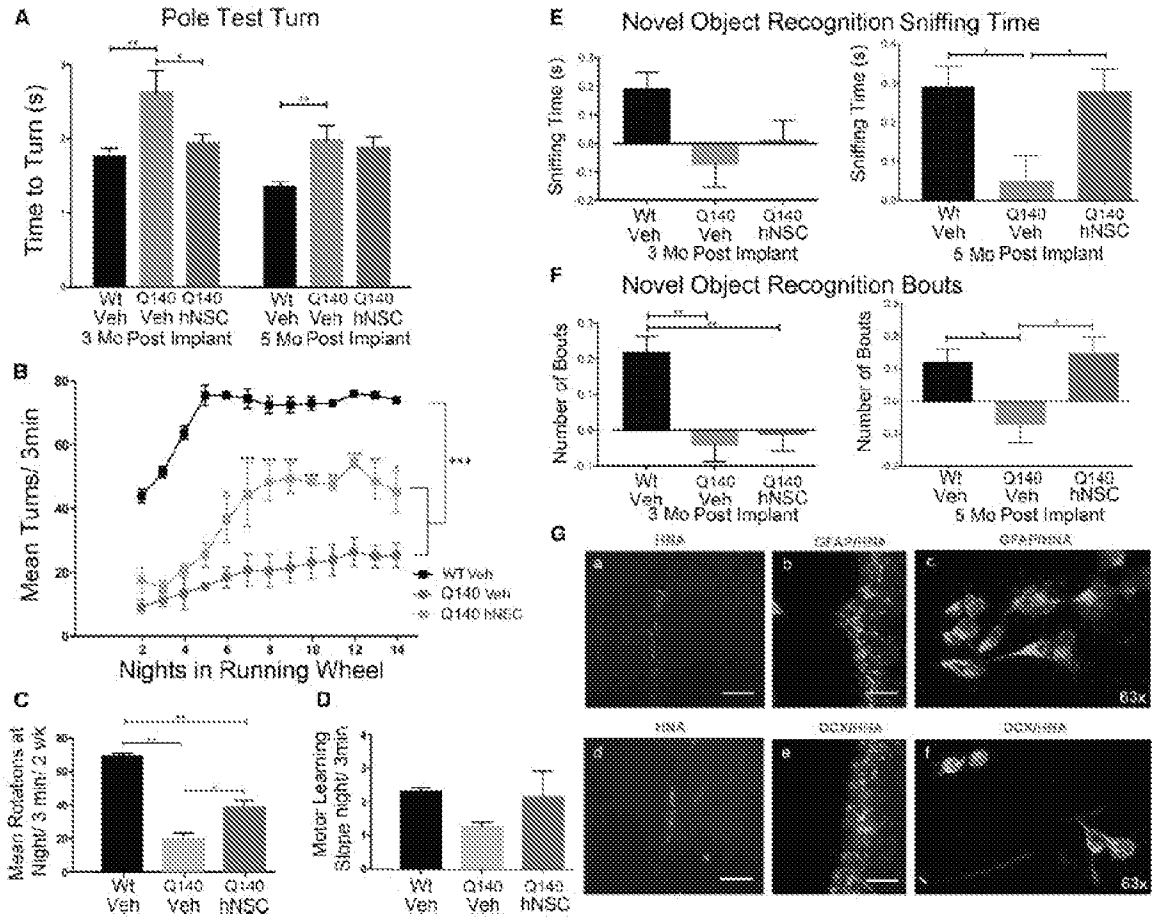
FIGS. 2A – 2F



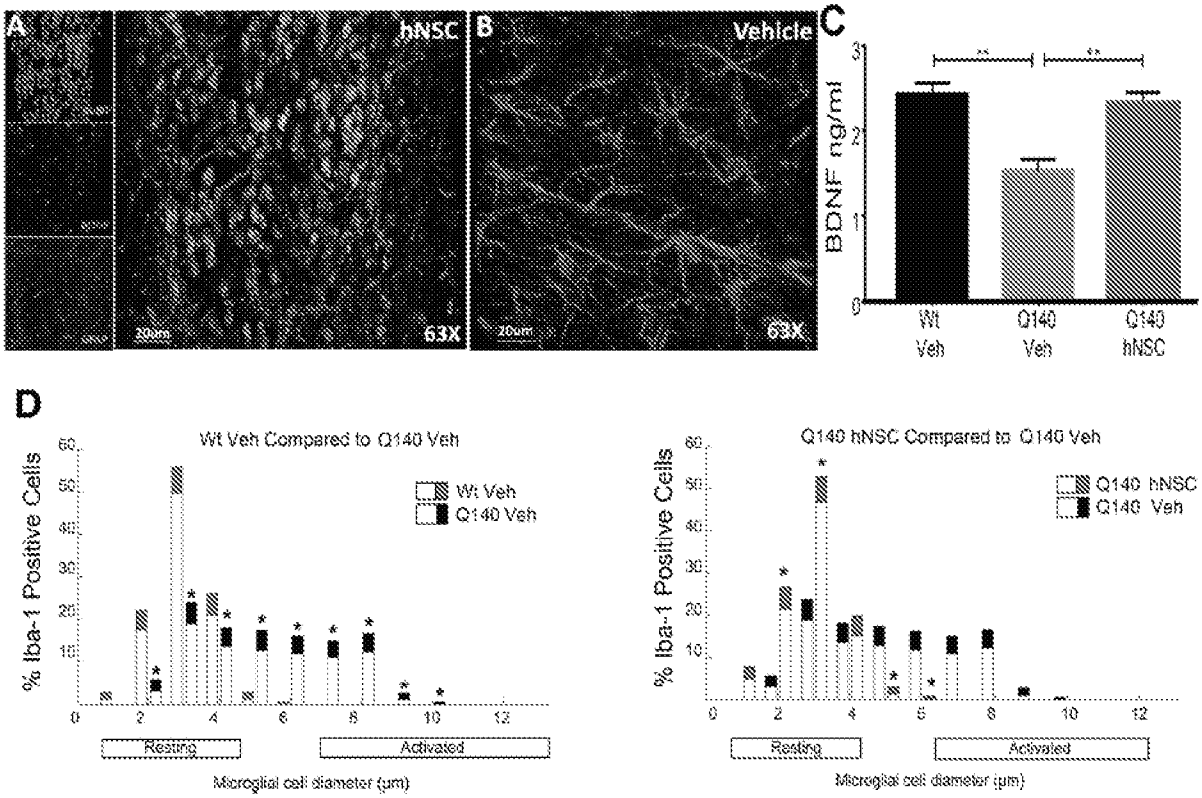
FIGS. 3A – 3F



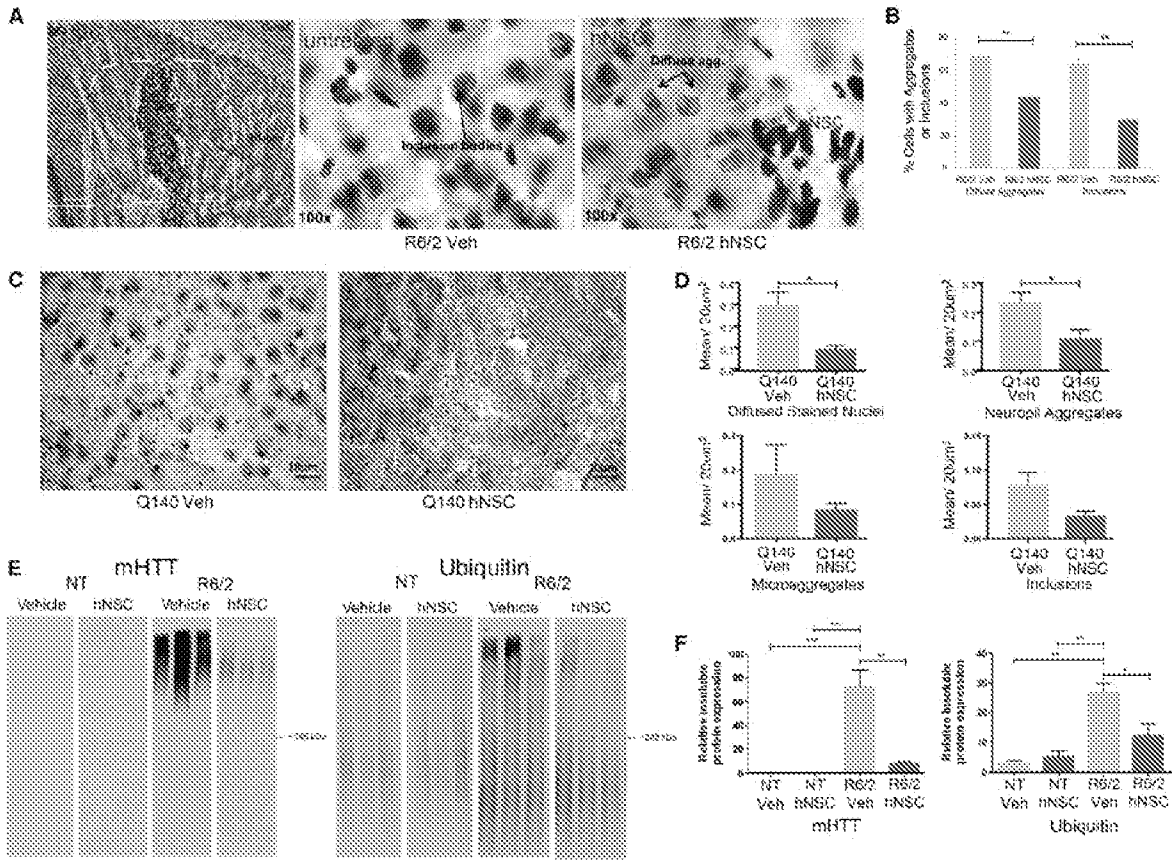
FIGS. 4A – 4B



FIGS. 5A – 5G


A
hNSC
63X
B
Vehicle
63X
C
BDNF ng/ml
Wt Veh
Q140 Veh
Q140 hNSC
D
Wt Veh Compared to Q140 Veh
% Iba-1 Positive Cells
Wt Veh
Q140 Veh
Resting
Activated
Microglial cell diameter (µm)
Q140 hNSC Compared to Q140 Veh
Q140 hNSC
Q140 Veh

FIGS. 6A – 6D


A
R6/2 Veh
R6/2 hNSC
B
C
Q140 Veh
Q140 hNSC
D
Q140 Veh
Q140 hNSC
Diffused Stained Nuclei
Neuropil Aggregates
Microaggregates
Inclusions
E
mHTT
Ubiquitin
NT
R6/2
Vehicle
hNSC
F
mHTT
Ubiquitin

FIGS. 7A – 7F

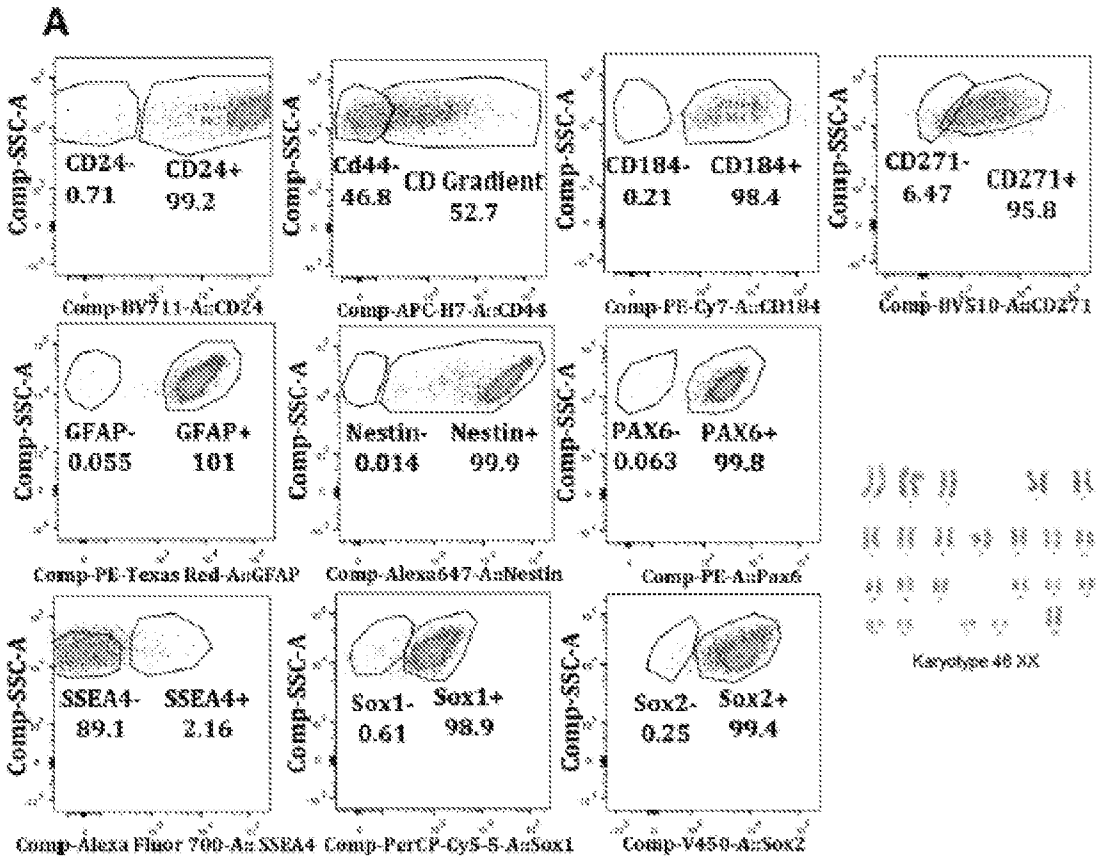

A
Comp-SSC-A
CD24-
0.71
CD24+
99.2
Comp-BV711-A::CD24
Cd44-
46.8
CD Gradient
52.7
Comp-APC-H7-A::CD44
CD184-
0.21
CD184+
98.4
Comp-PE-Cy7-A::CD184
CD271-
6.47
CD271+
95.8
Comp-BV510-A::CD271
GFAP-
0.055
GFAP+
101
Comp-PE-Texas Red-A::GFAP
Nestin-
0.014
Nestin+
99.9
Comp-Alexa647-A::Nestin
PAX6-
0.063
PAX6+
99.8
Comp-PE-A::Pax6
SSEA4-
89.1
SSEA4+
2.16
Comp-Alexa Fluor 700-A::SSEA4
Sox1-
0.61
Sox1+
98.9
Comp-PerCP-Cy5-5-A::Sox1
Sox2-
0.25
Sox2+
99.4
Comp-V450-A::Sox2
Karyotype 46 XX

FIG. 8A

B

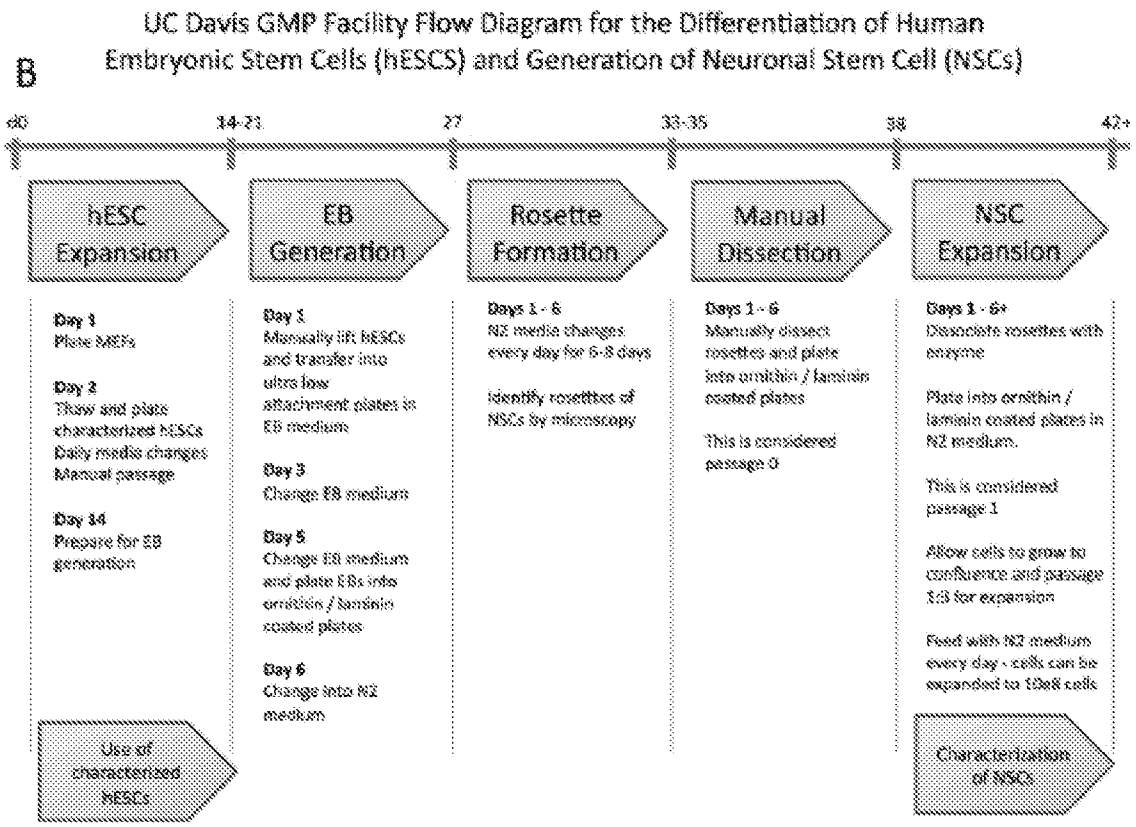


FIG. 8B

C

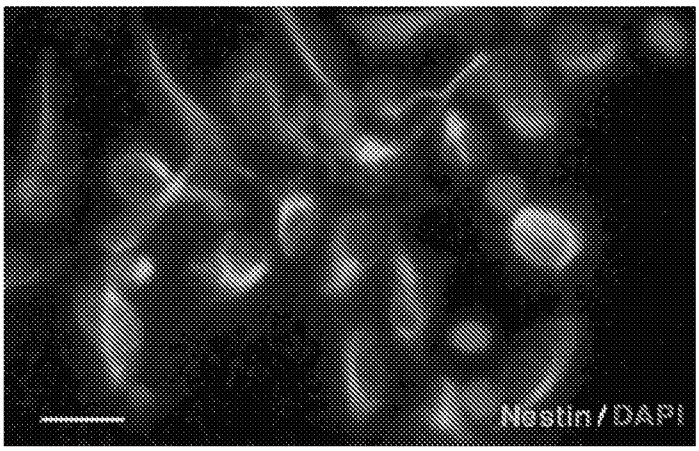


FIG. 8C

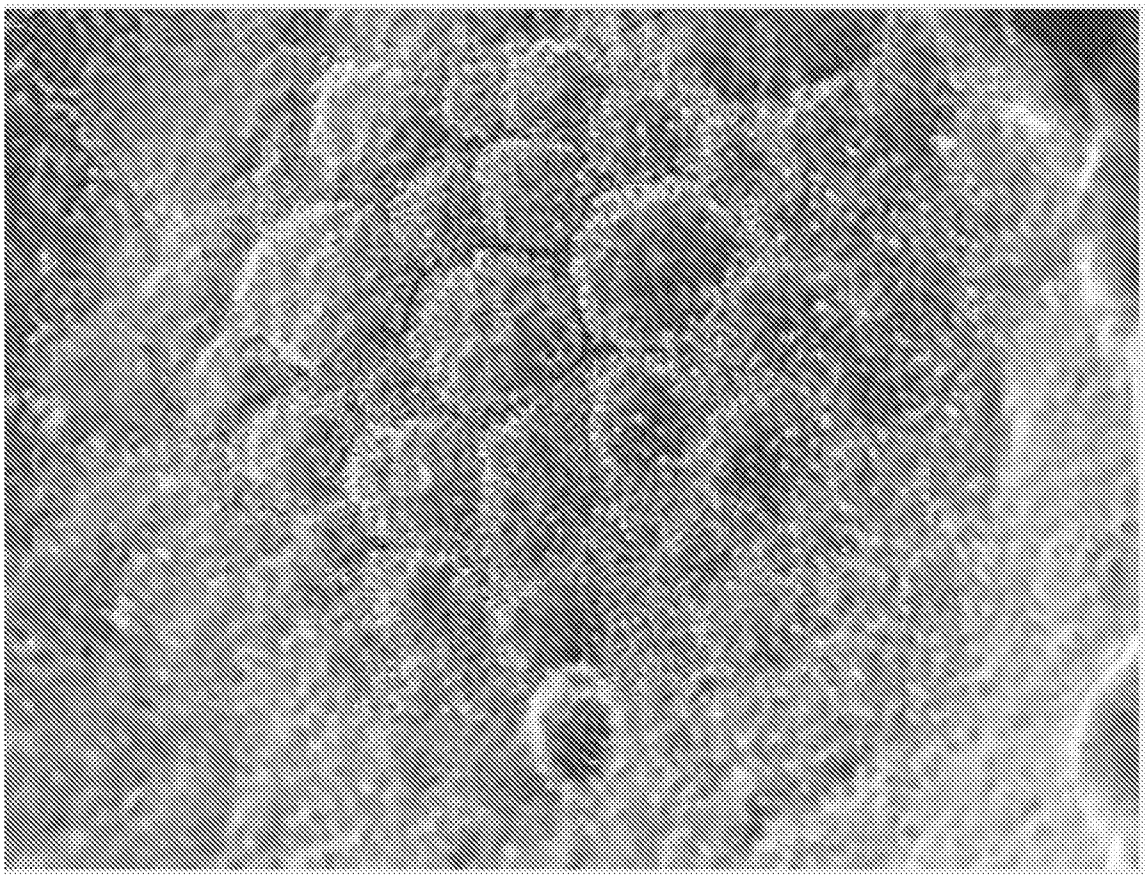


FIG. 8D

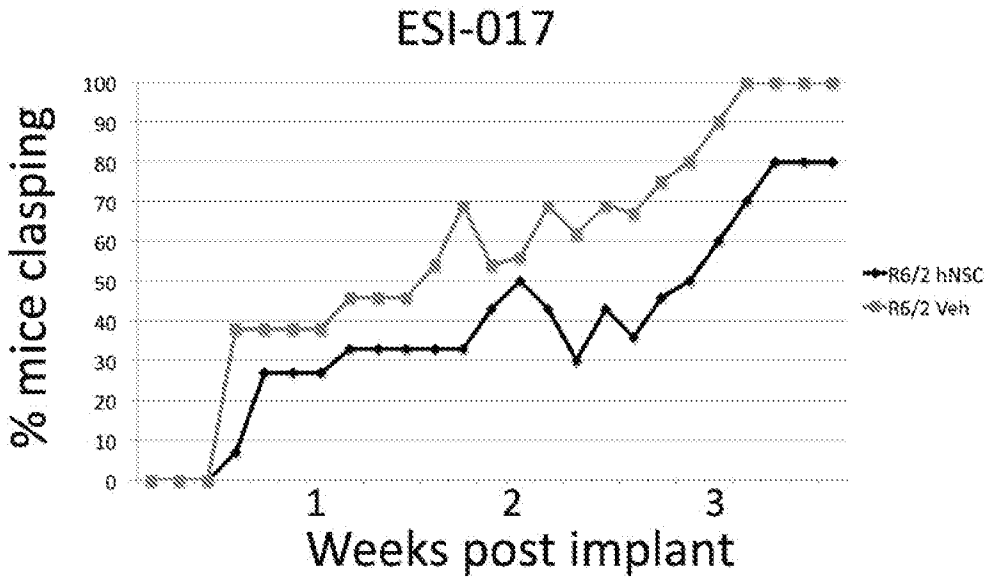
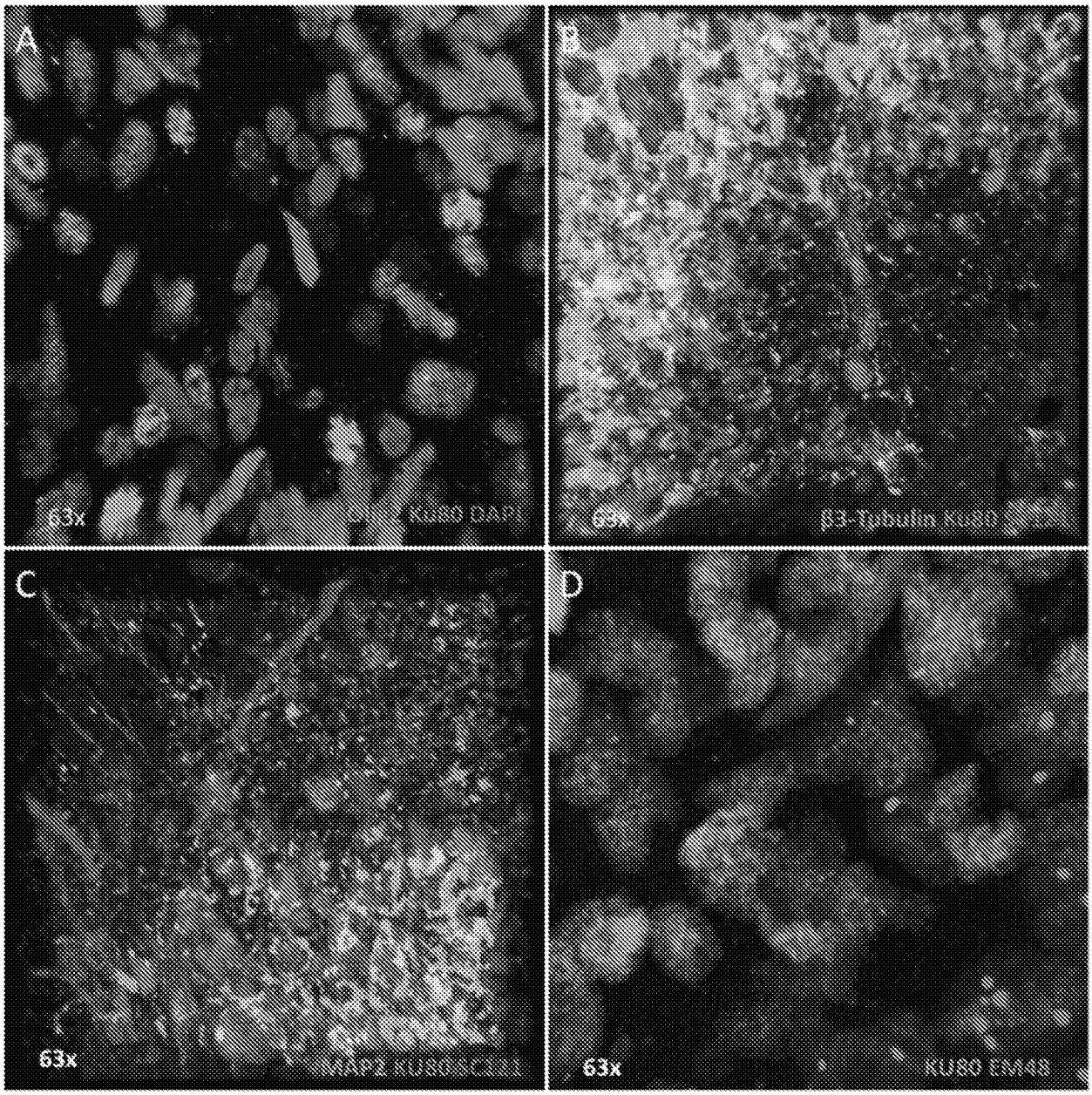

ESI-017
% mice clasping
100
90
80
70
60
50
40
30
20
10
0
1
2
3
Weeks post implant
R6/2 hNSC
R6/2 Veh

FIG. 9



FIGS. 10A – 10D

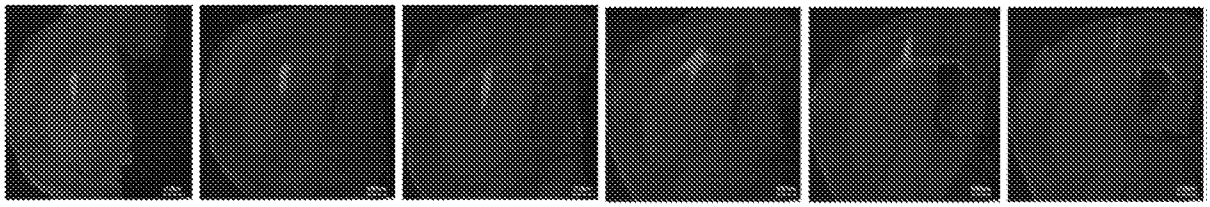
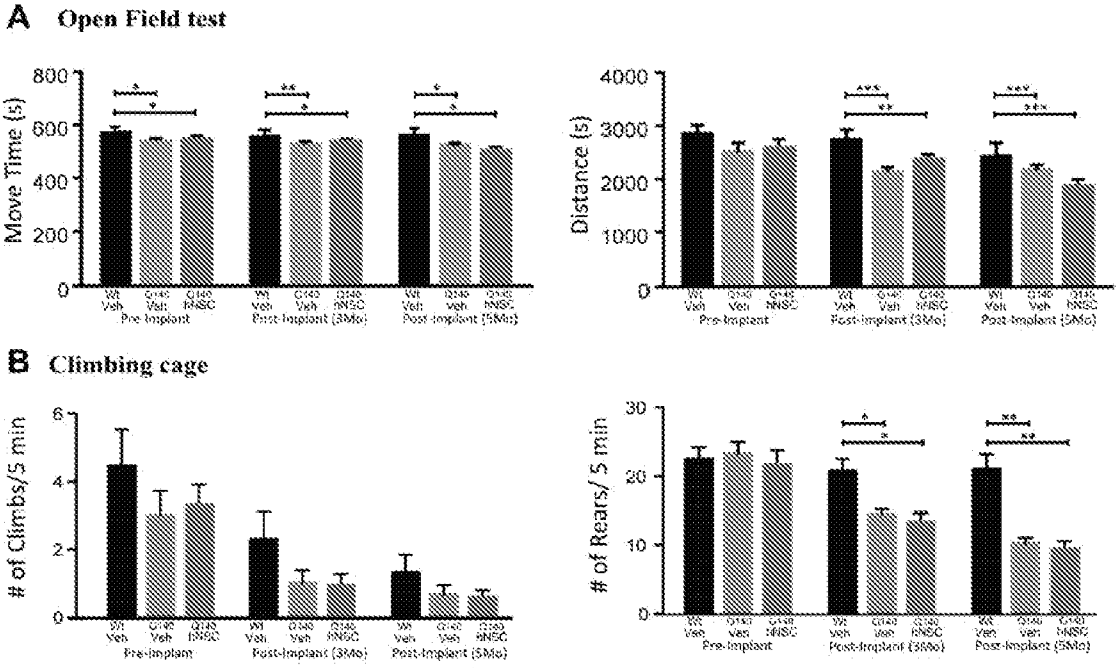
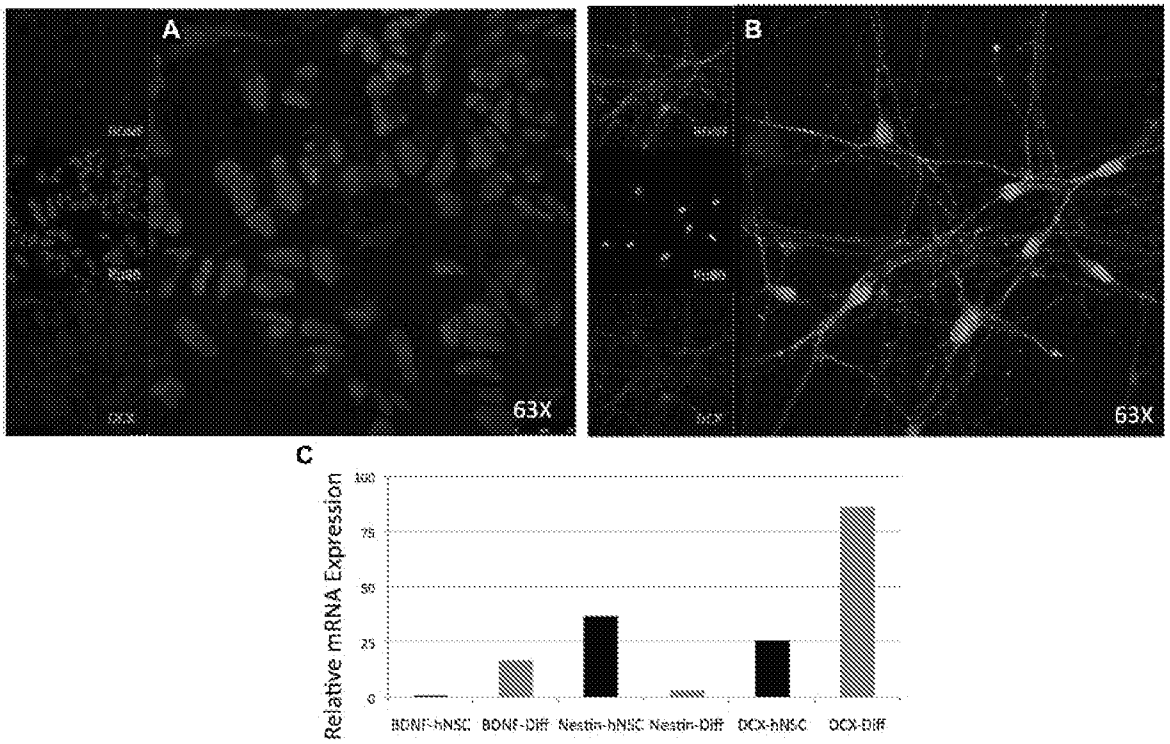


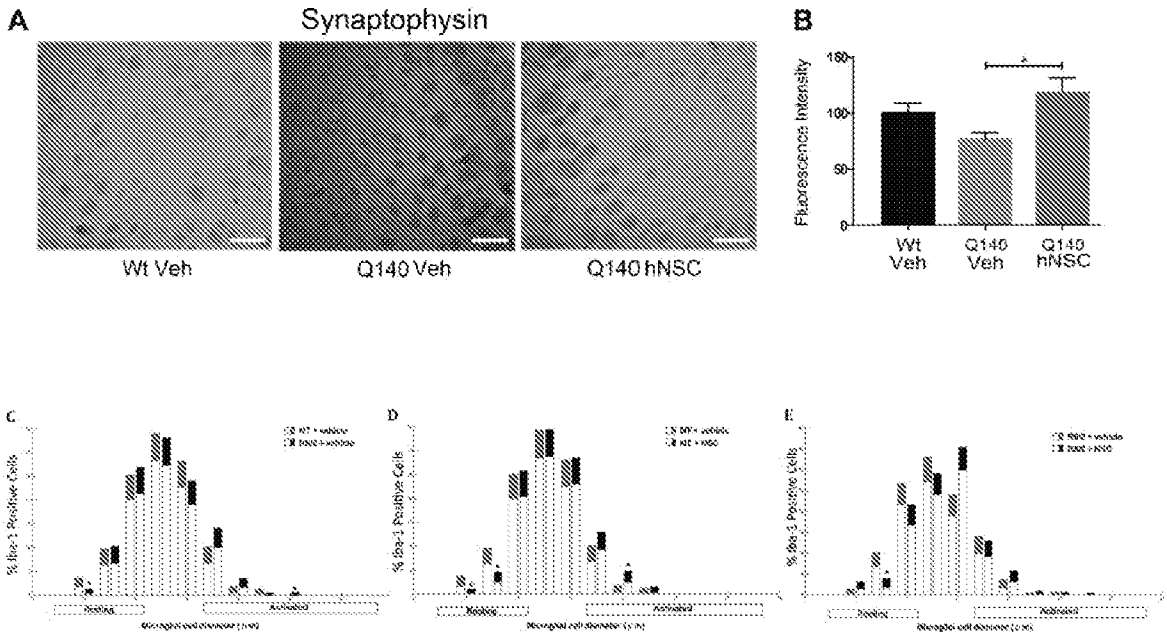
FIG. 10E



FIGS. 11A – 11B



FIGS. 12A – 12C



FIGS. 13A – 13E

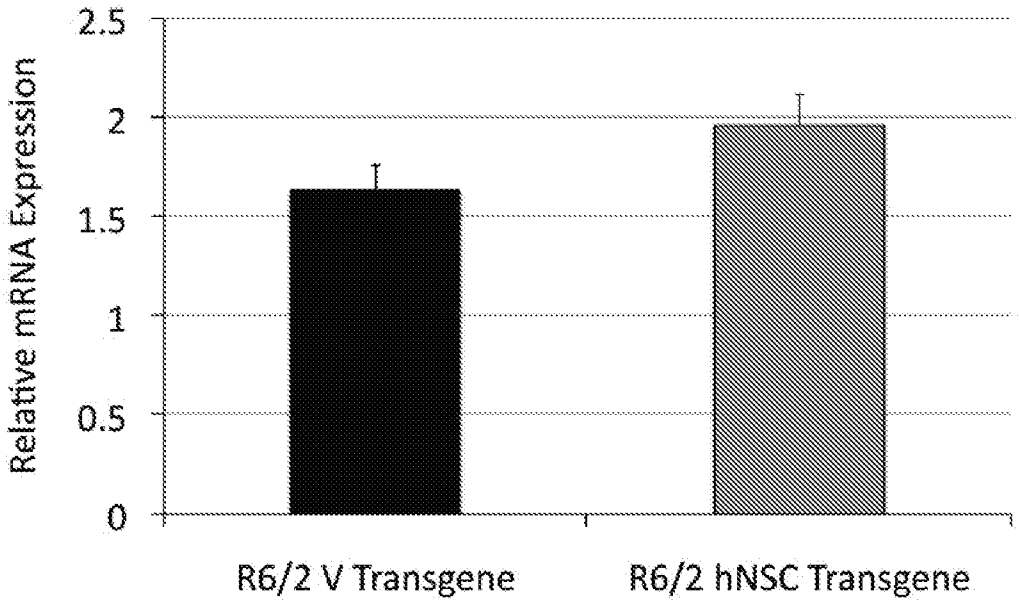
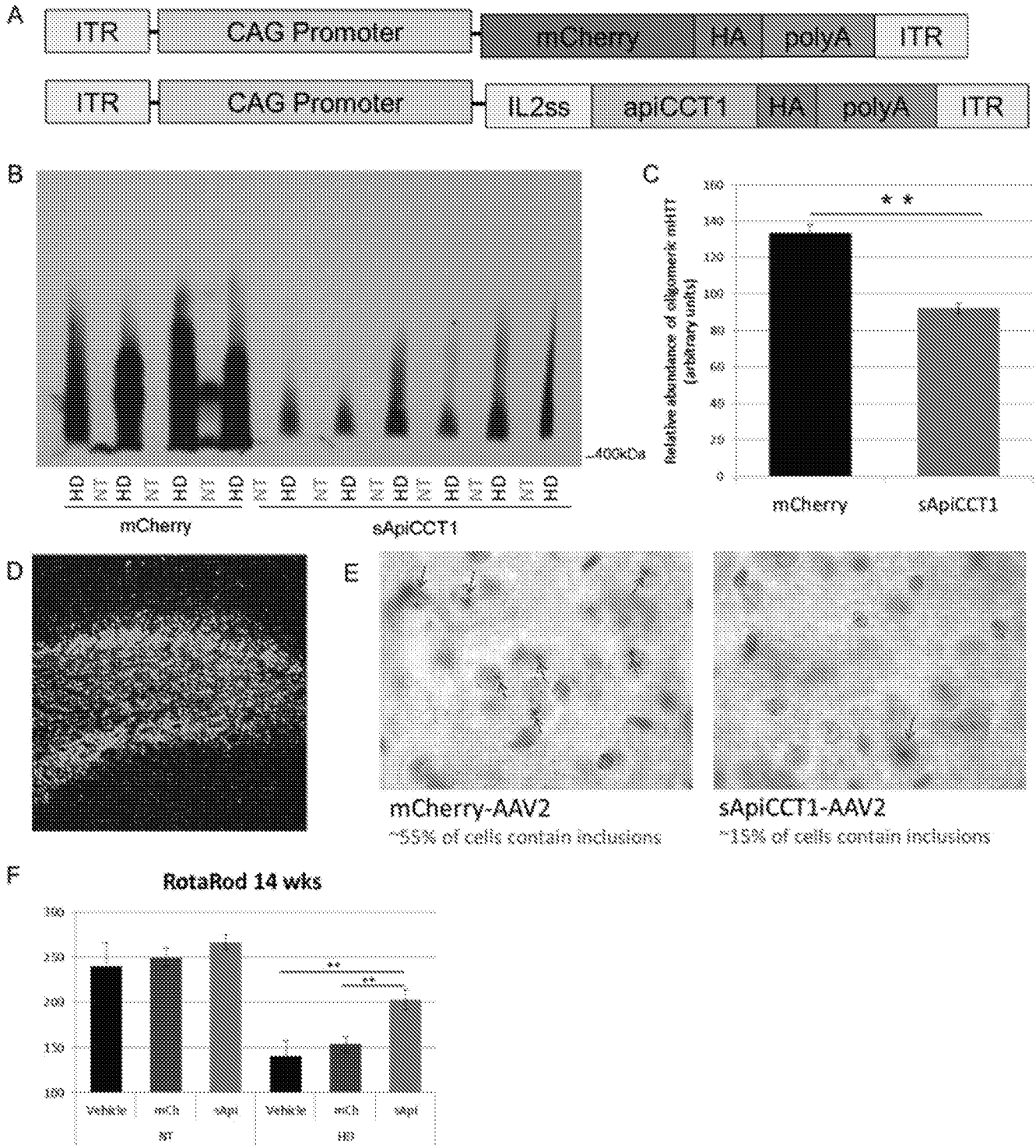
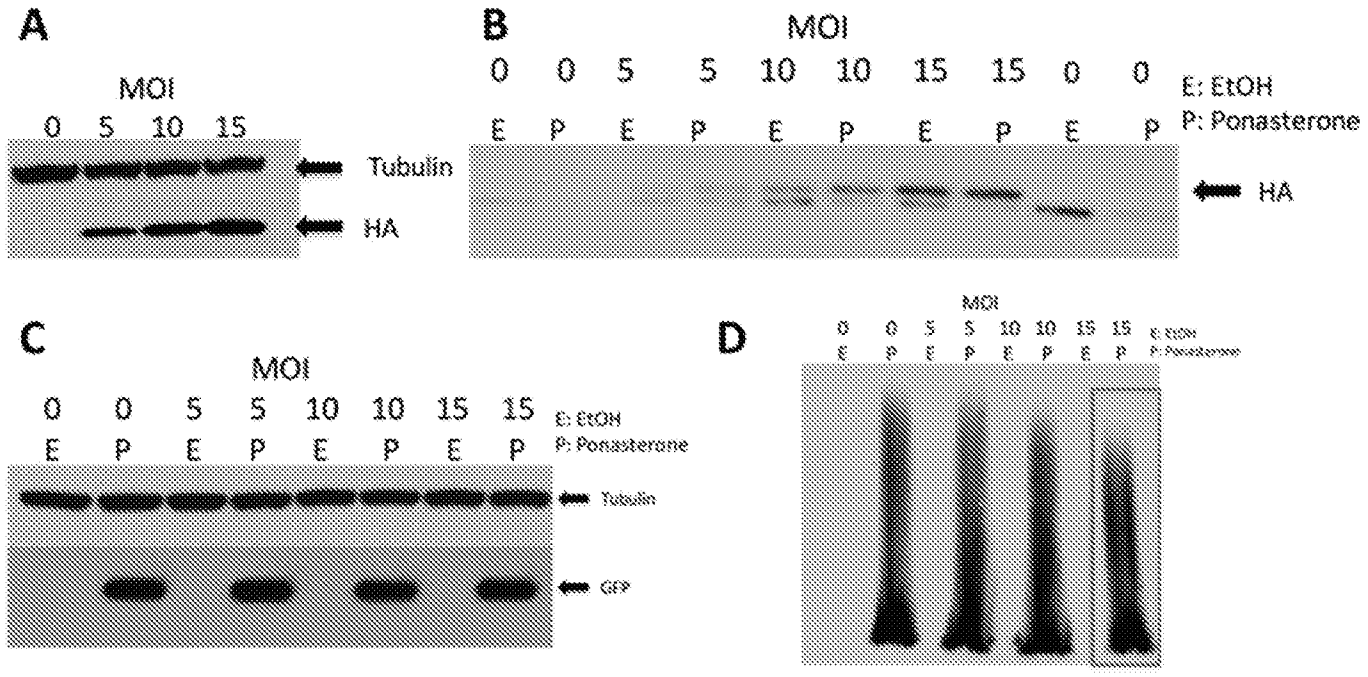


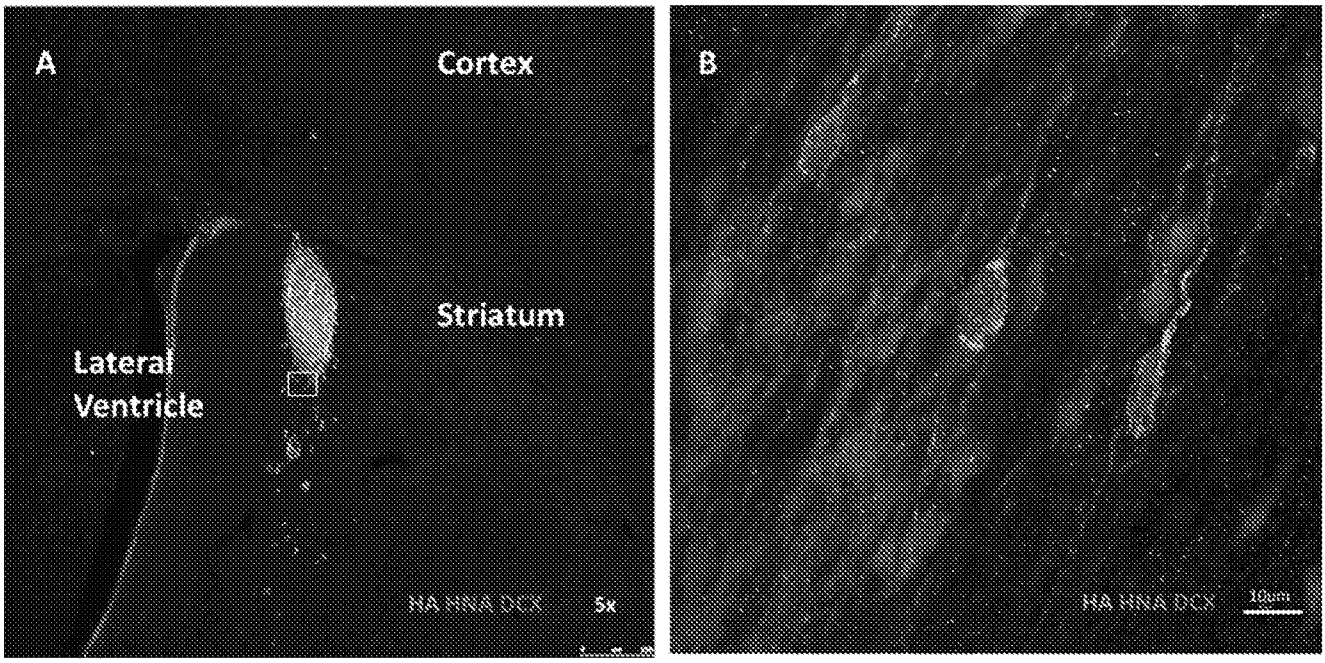
FIG. 14


A
ITR
CAG Promoter
mCherry
HA
polyA
ITR
ITR
CAG Promoter
IL2ss
apiCCT1
HA
polyA
ITR
B
~400kDa
mCherry
sApiCCT1
C
Relative abundance of oligomeric mHTT (arbitrary units)
**
mCherry
sApiCCT1
D
E
mCherry-AAV2
~55% of cells contain inclusions
sApiCCT1-AAV2
~15% of cells contain inclusions
F
RotaRod 14 wks
Vehicle
mCh
sApi
Vehicle
mCh
sApi
ST
HD

FIGS. 15A – 15F


A
MOI
0 5 10 15
Tubulin
HA
B
MOI
0 0 5 5 10 10 15 15 0 0
E P E P E P E P E P
E: EtOH
P: Ponasterone
HA
C
MOI
0 0 5 5 10 10 15 15
E P E P E P E P
E: EtOH
P: Ponasterone
Tubulin
GFP
D
MOI

FIGS. 16A – 16D


A
Cortex
Striatum
Lateral
Ventricle
HA HNA DCX
5x
B
HA HNA DCX
10um

FIGS. 17A – 17B