


Figure 1

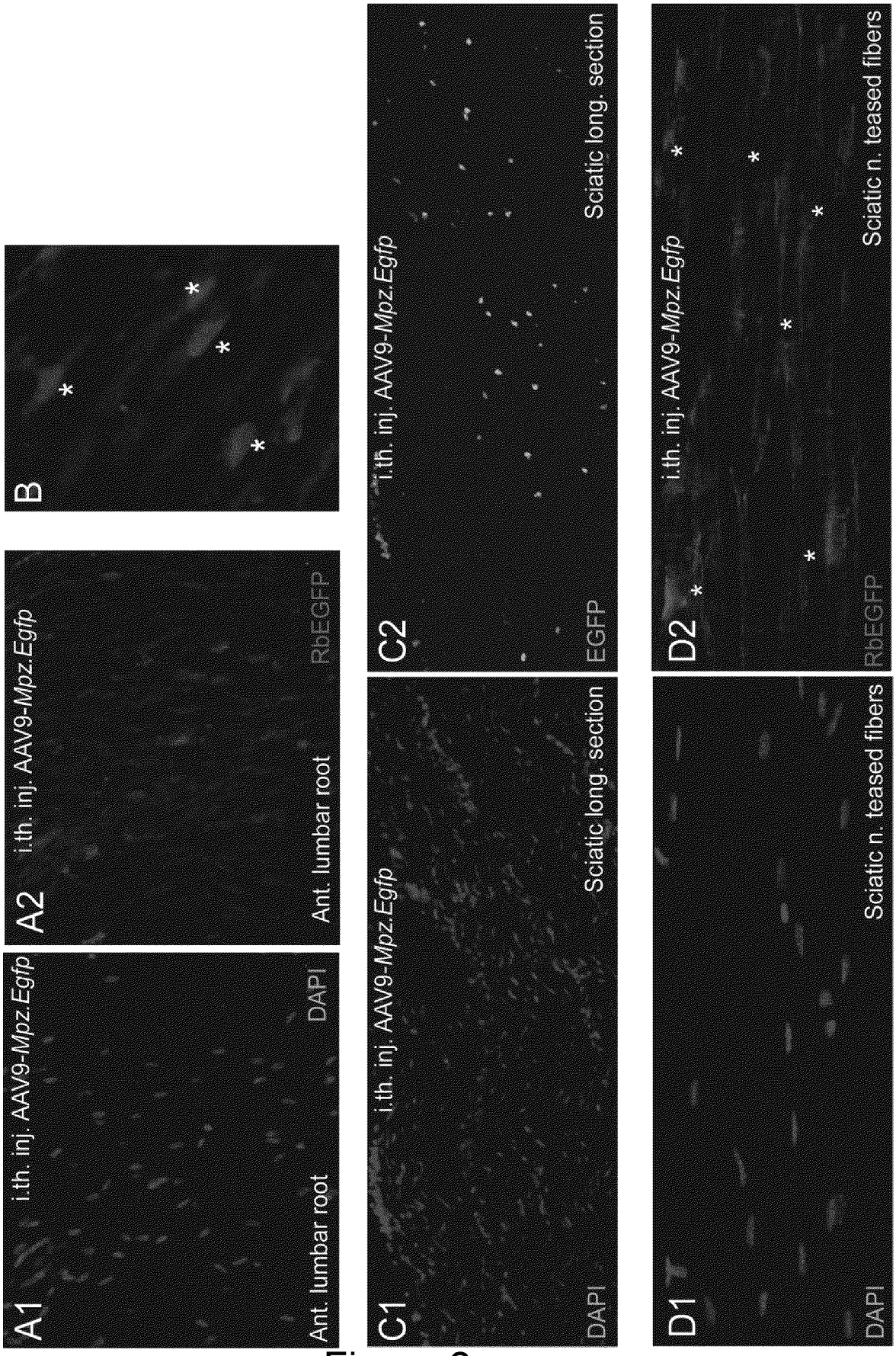
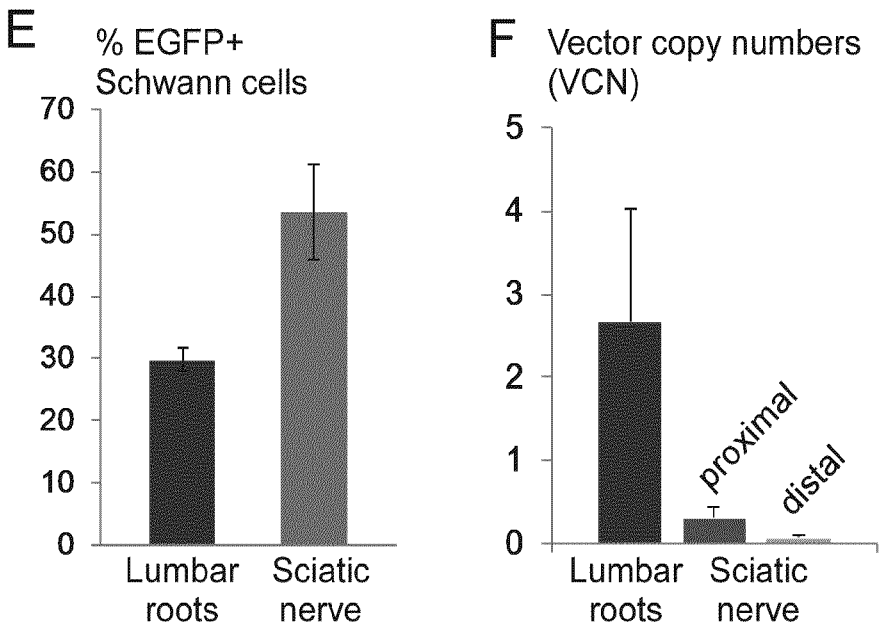



Figure 2
(part 1/2)



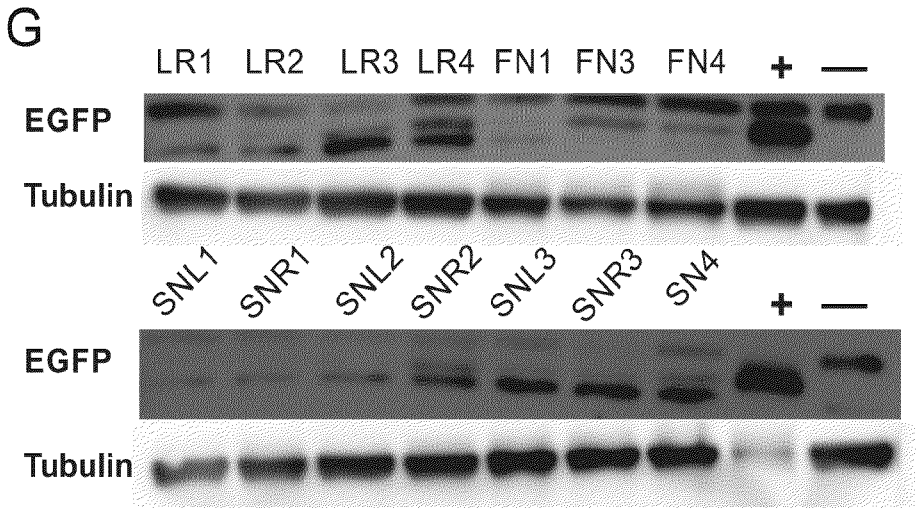




Figure 2
(part 2/2)

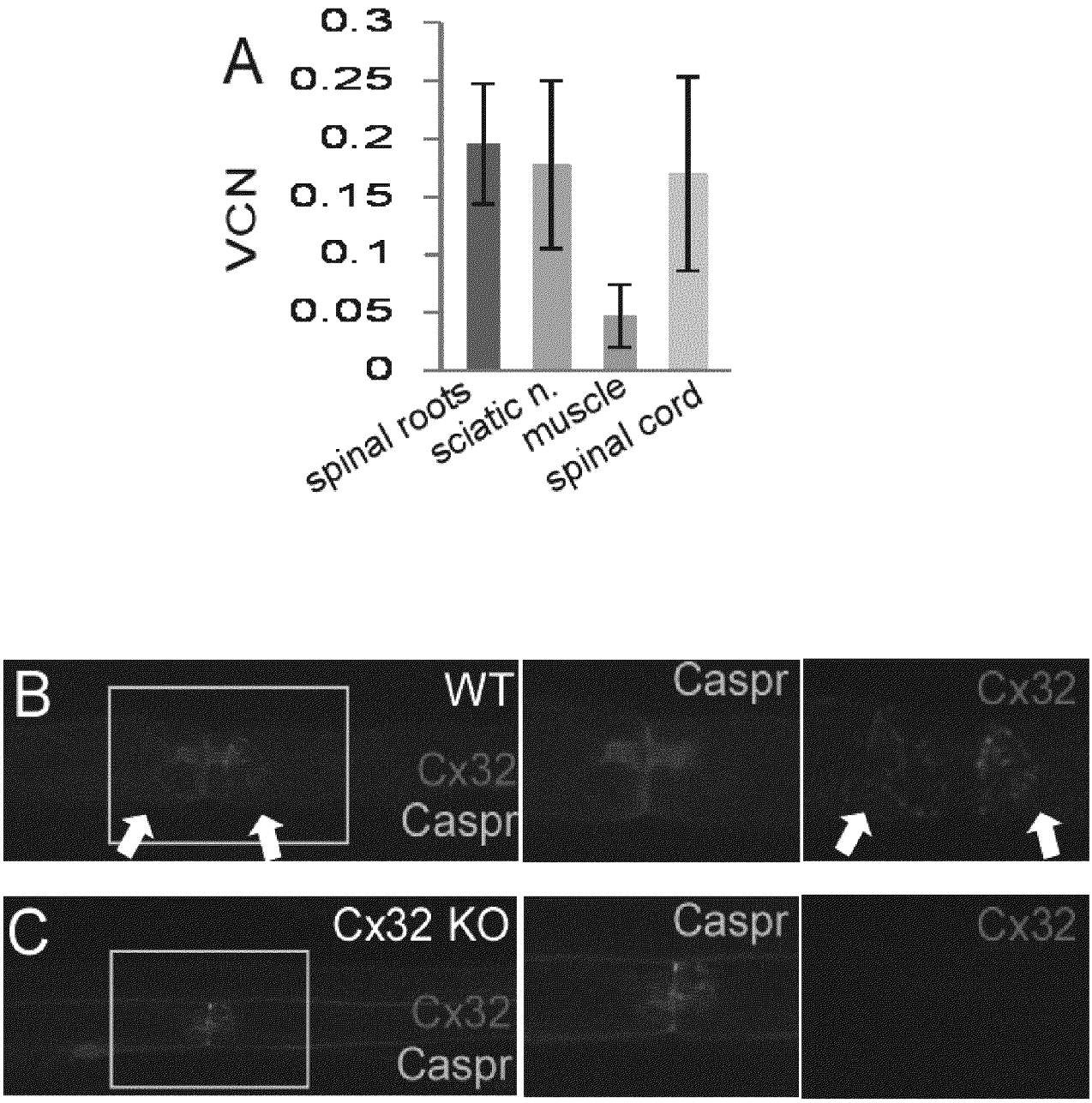



Figure 3
(part 1/2)

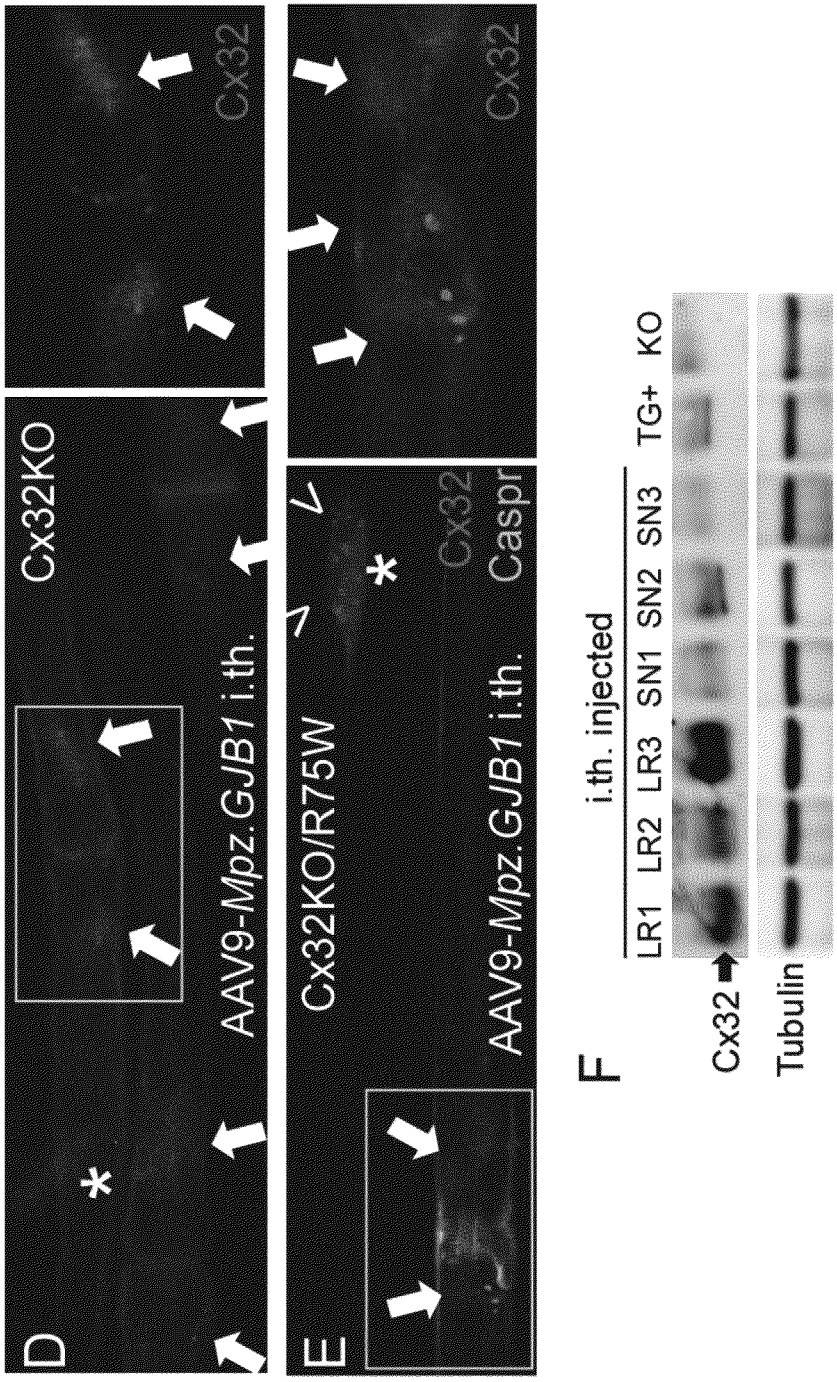
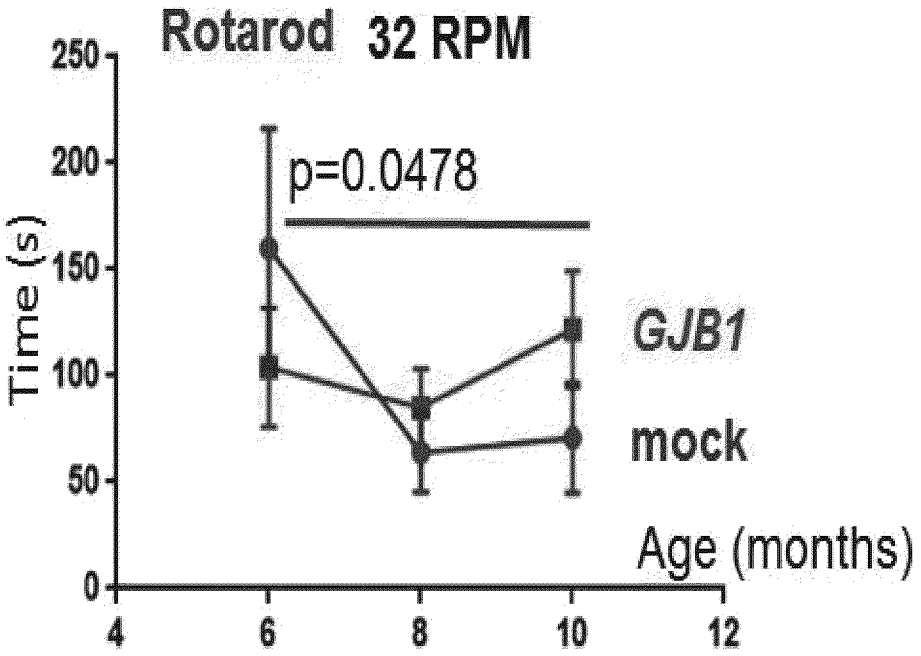



Figure 3
(part 2/2)


Rotarod 32 RPM
Time (s)
250
200
150
100
50
0
p=0.0478
GJB1
mock
Age (months)
4
6
8
10
12

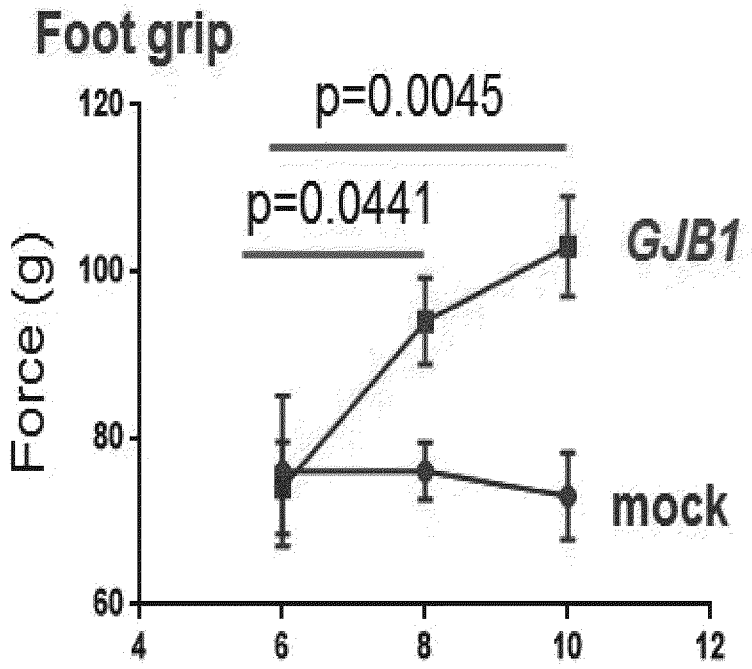

Foot grip
Force (g)
120
100
80
60
p=0.0045
p=0.0441
GJB1
mock
4
6
8
10
12

Figure 4

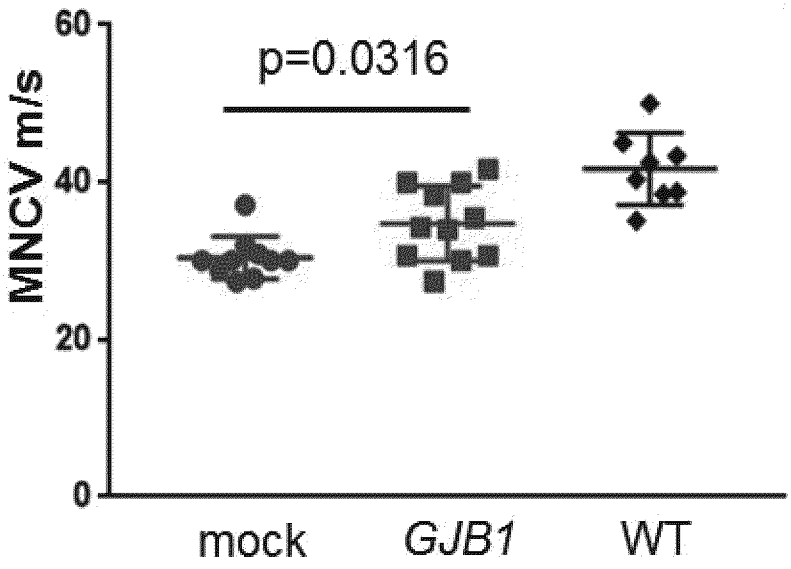



Figure 5

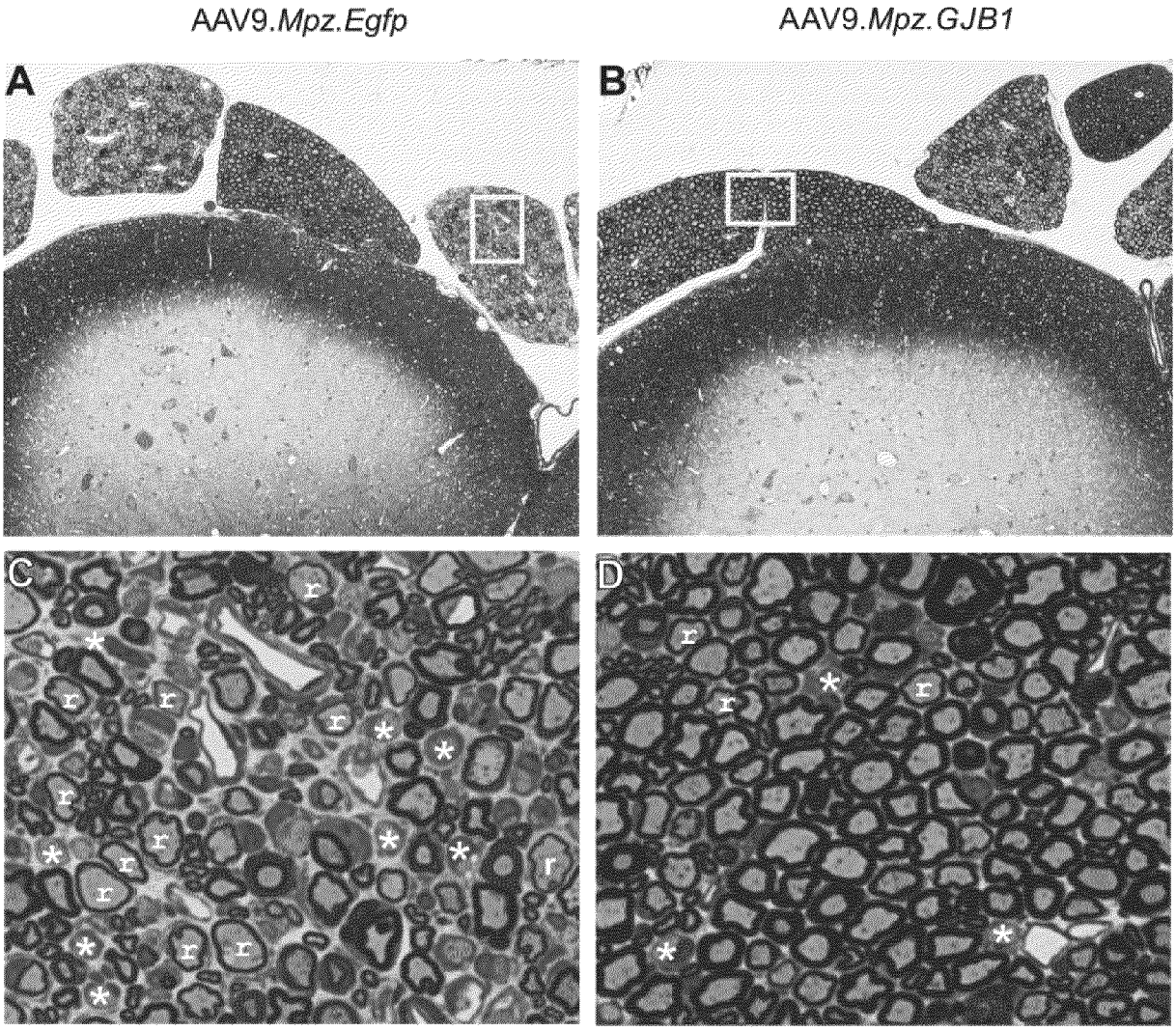
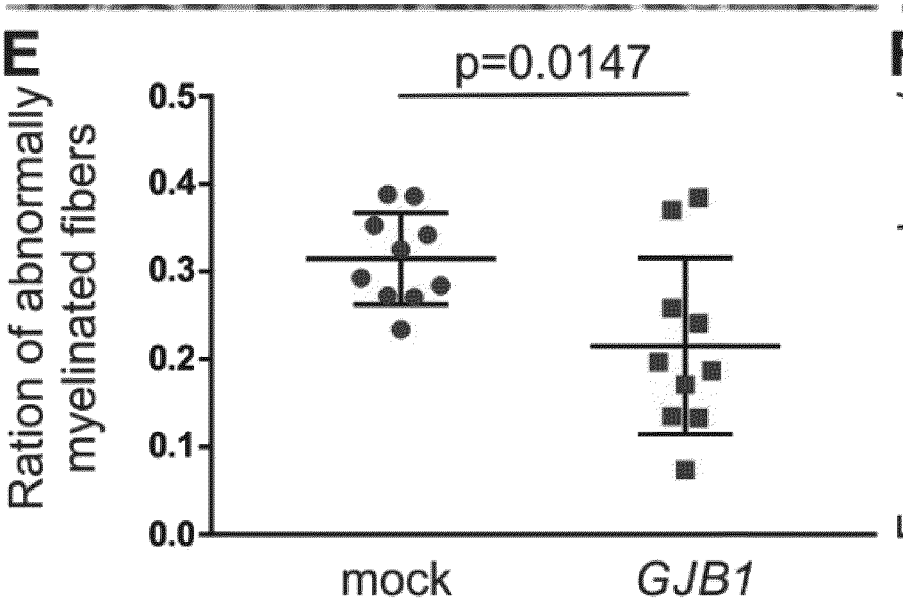



Figure 6
(part 1 of 2)



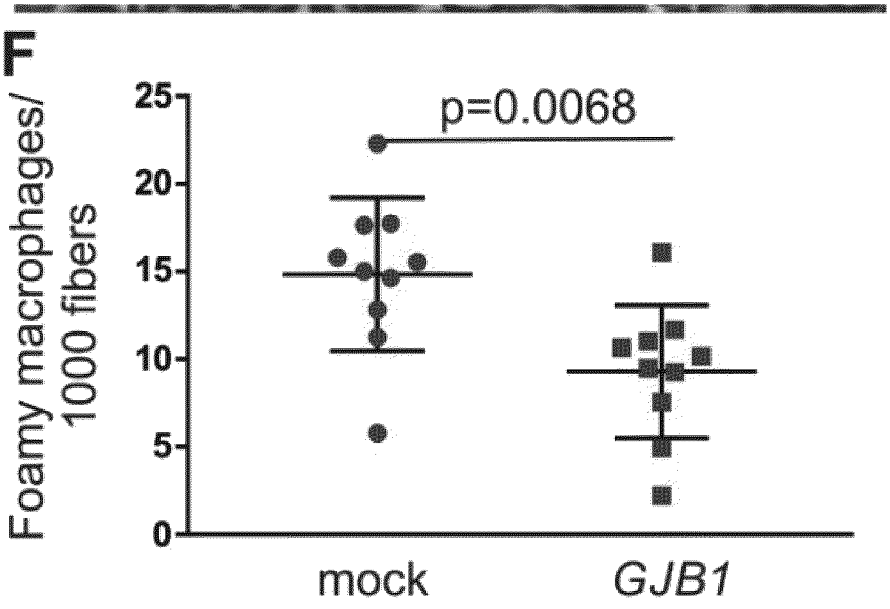




Figure 6
(part 2 of 2)

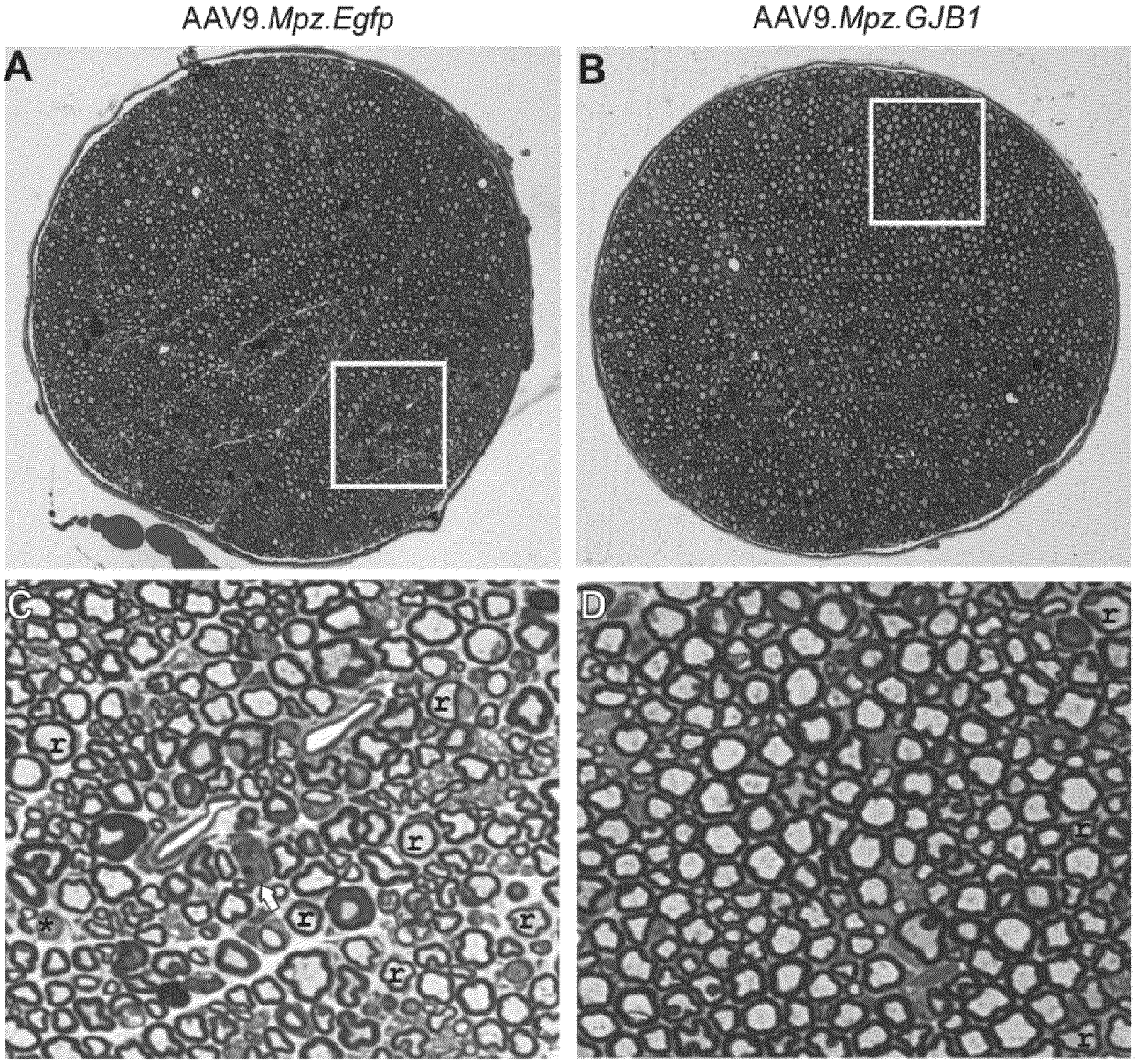



Figure 7
(part 1 of 2)

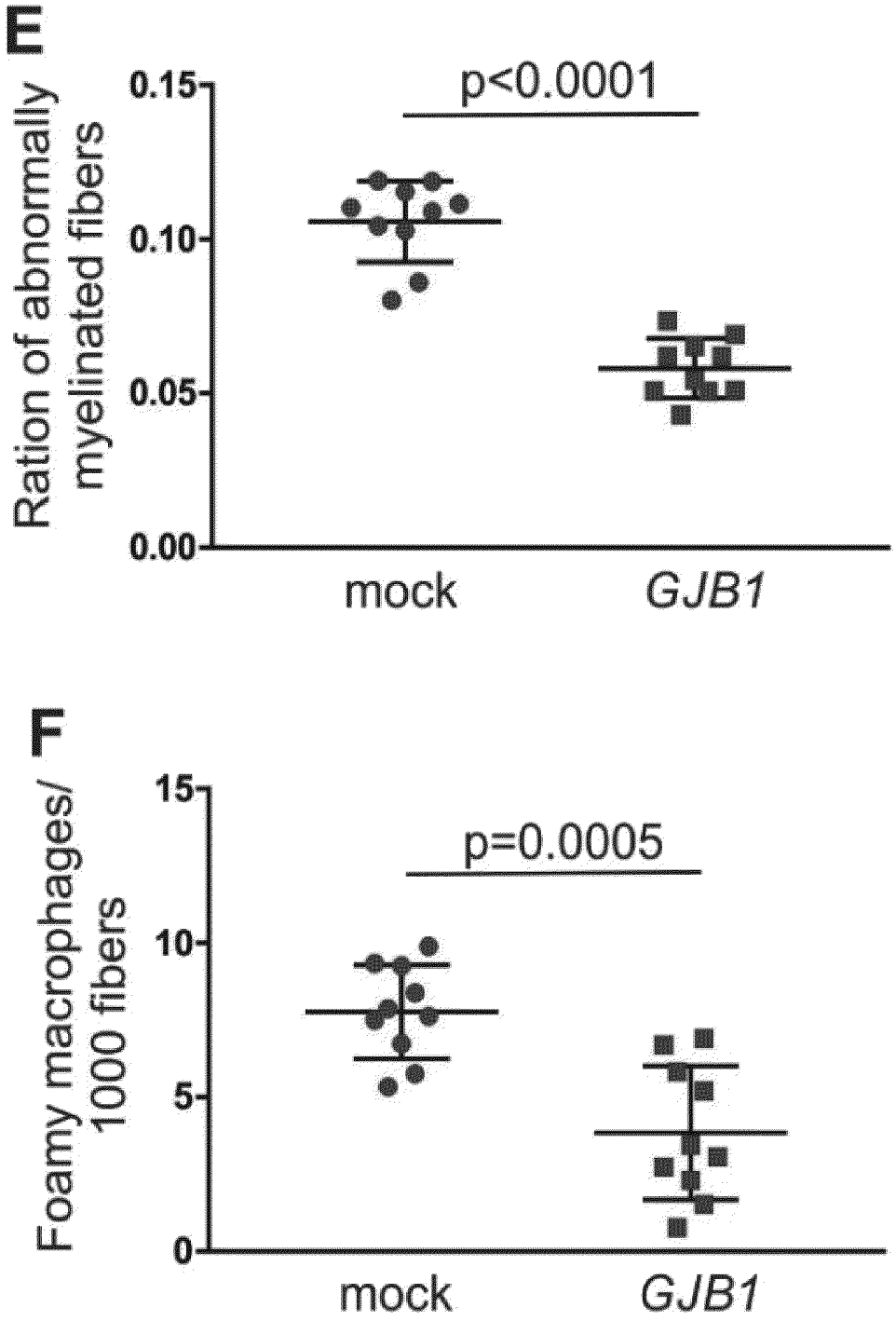



Figure 7
(part 2 of 2)

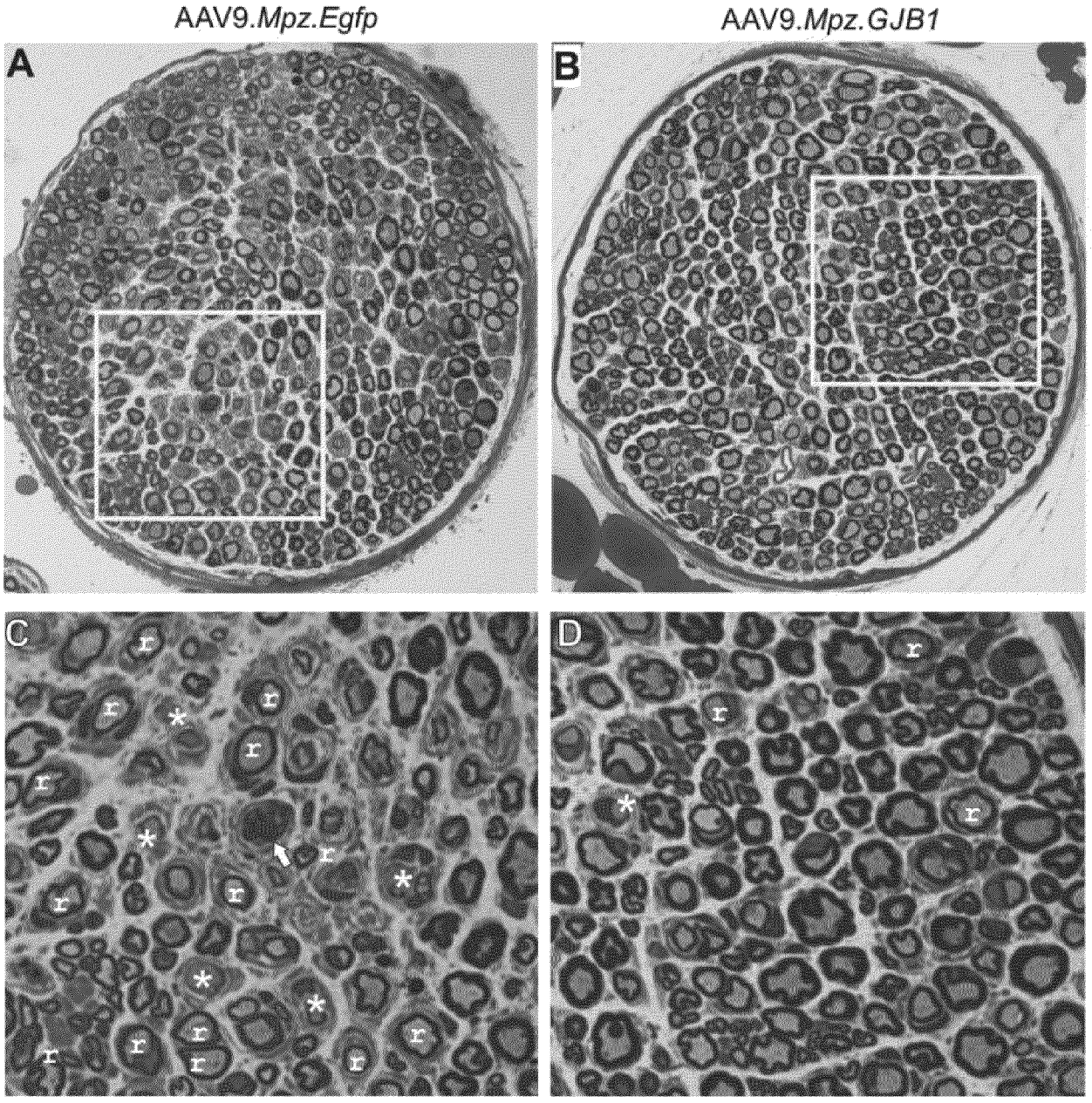



Figure 8
(part 1 of 2)

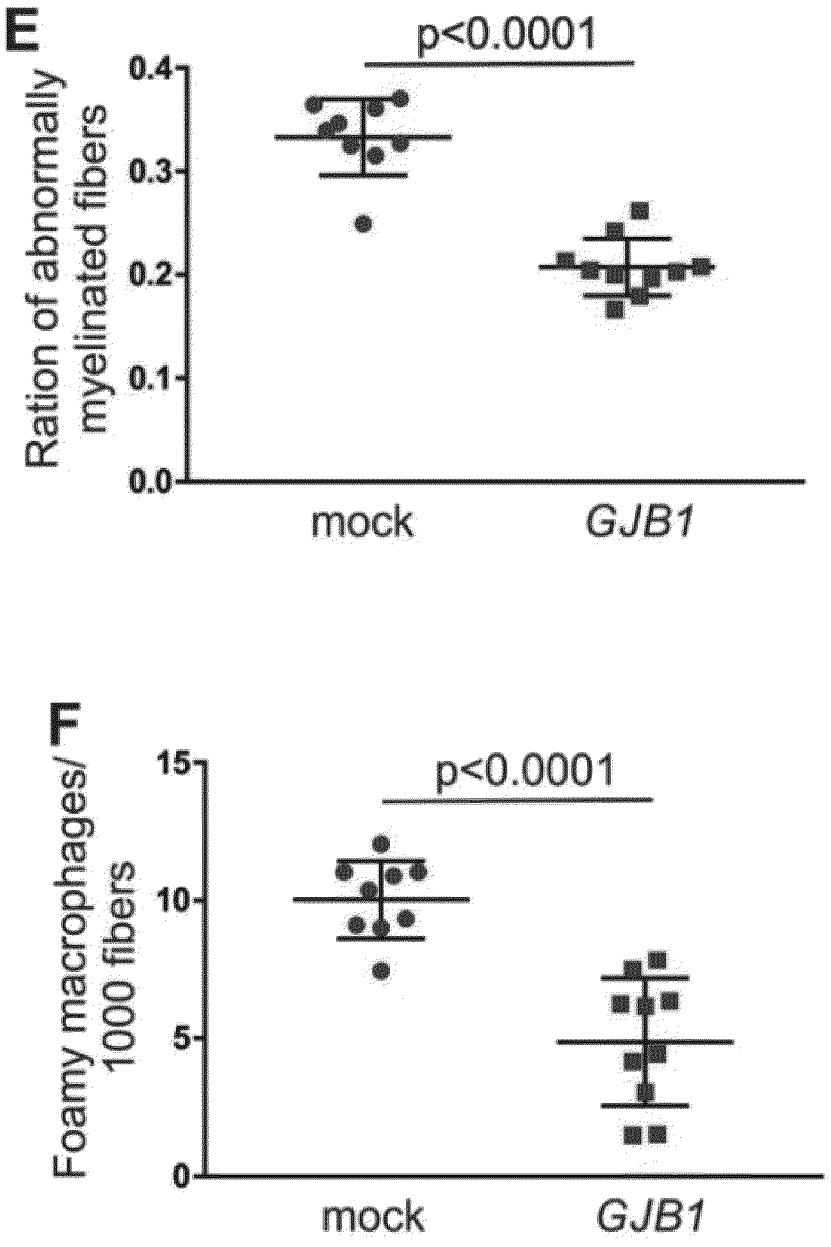



Figure 8
(part 2 of 2)

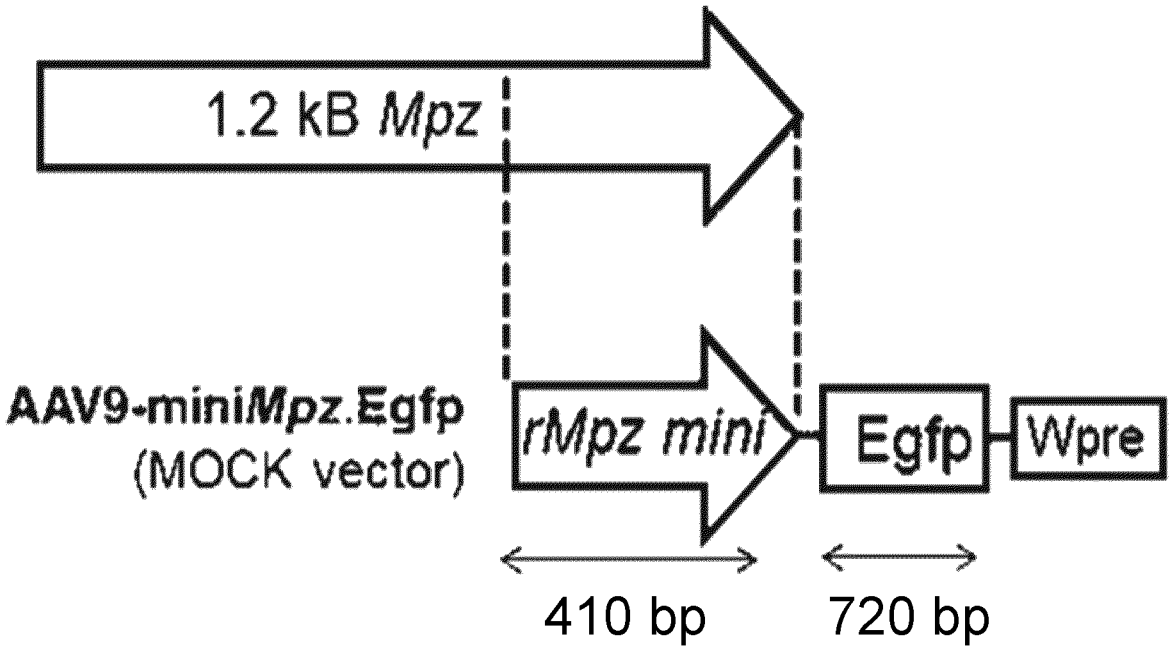

1.2 kB Mpz
AAV9-miniMpz.Egfp
(MOCK vector)
rMpz mini
Egfp
Wpre
410 bp
720 bp

Figure 9

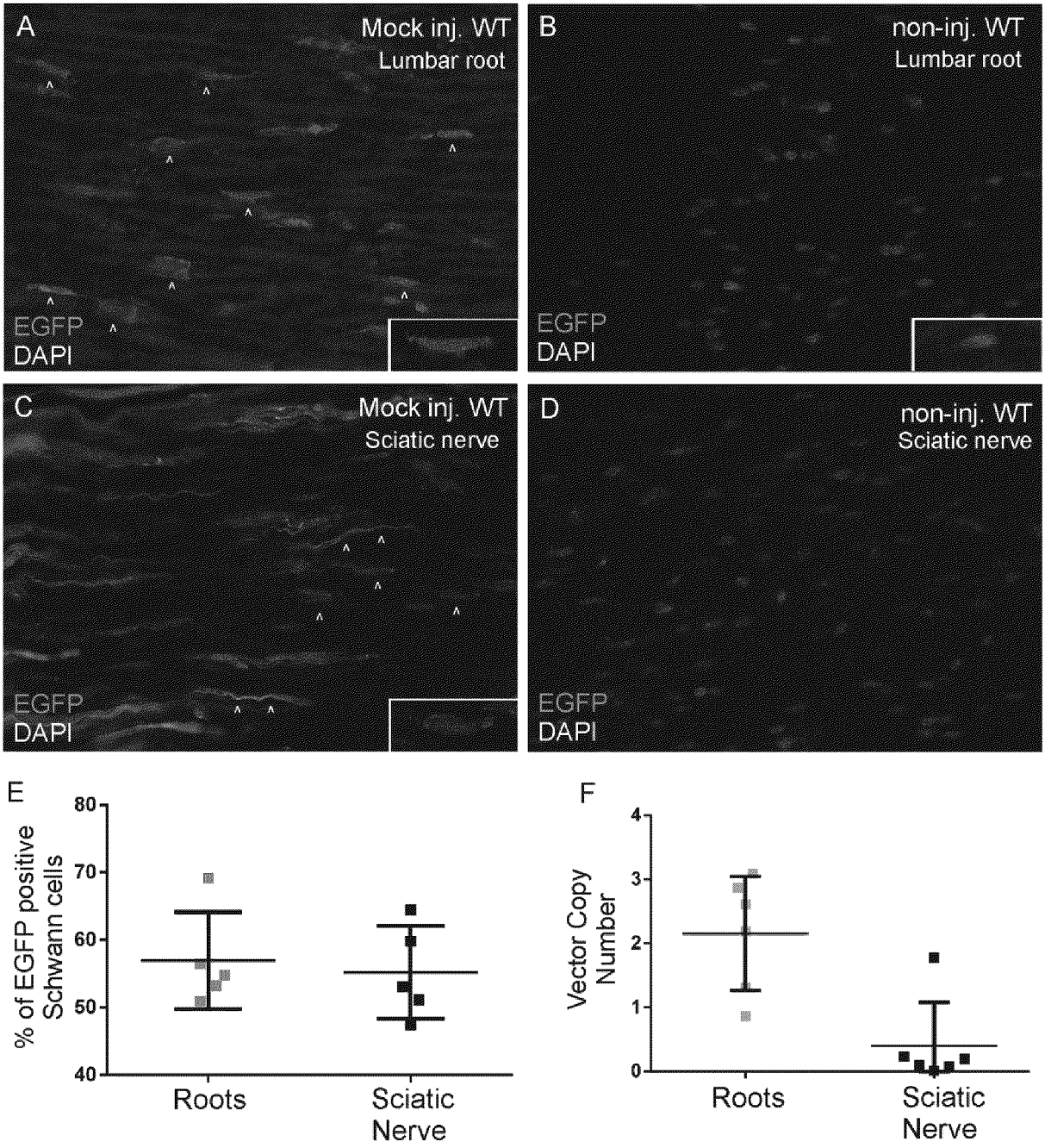

A
Mock inj. WT
Lumbar root
EGFP
DAPI
B
non-inj. WT
Lumbar root
EGFP
DAPI
C
Mock inj. WT
Sciatic nerve
EGFP
DAPI
D
non-inj. WT
Sciatic nerve
EGFP
DAPI
E
% of EGFP positive Schwann cells
80
70
60
50
40
Roots
Sciatic Nerve
F
Vector Copy Number
4
3
2
1
0
Roots
Sciatic Nerve

Figure 10

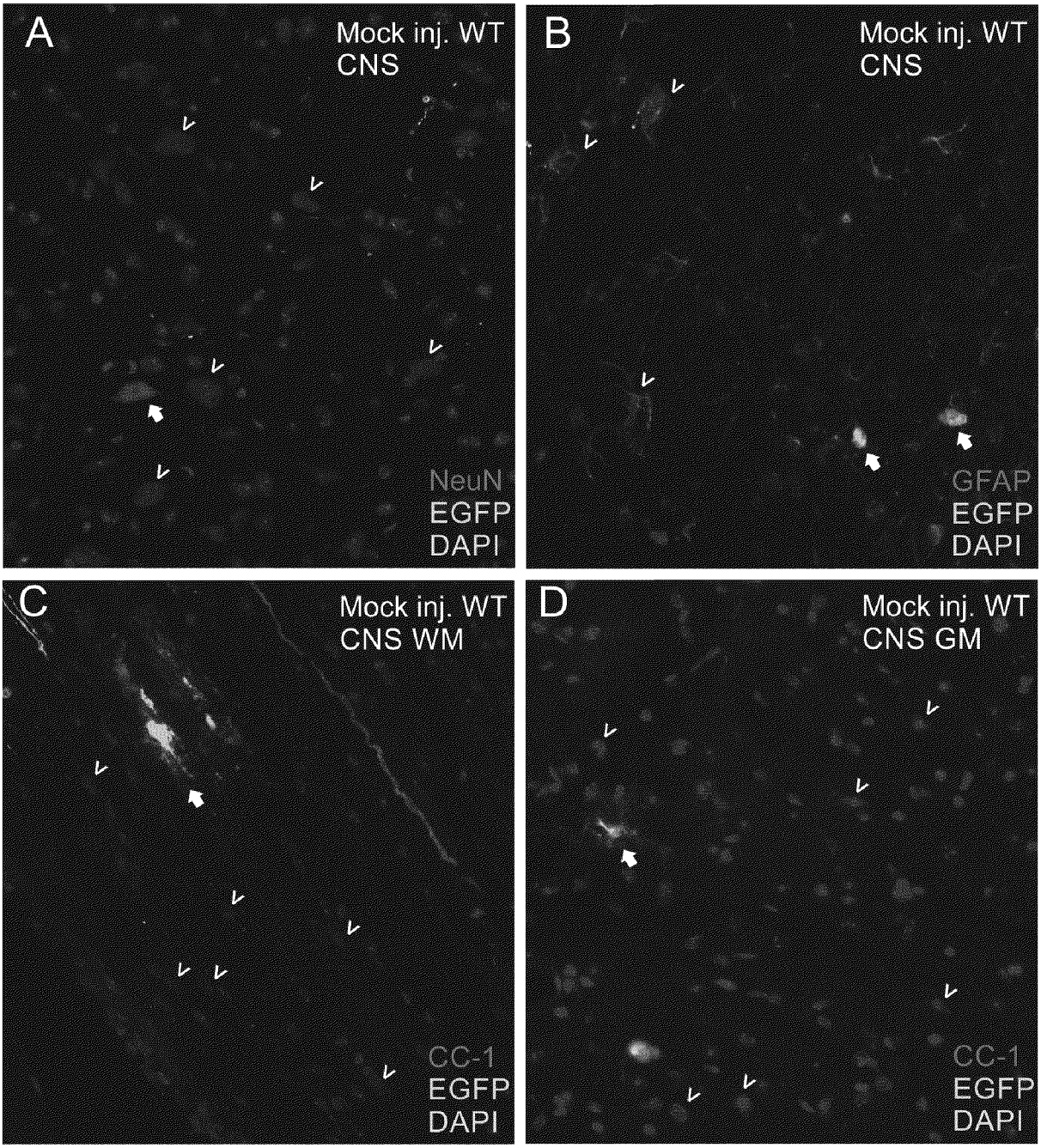



Figure 11
(part 1 of 2)

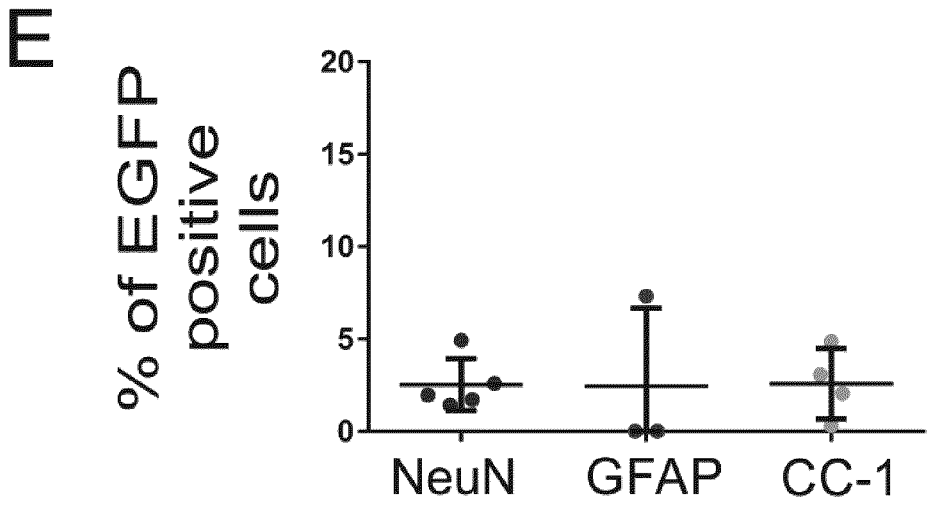



Figure 11
(part 2 of 2)

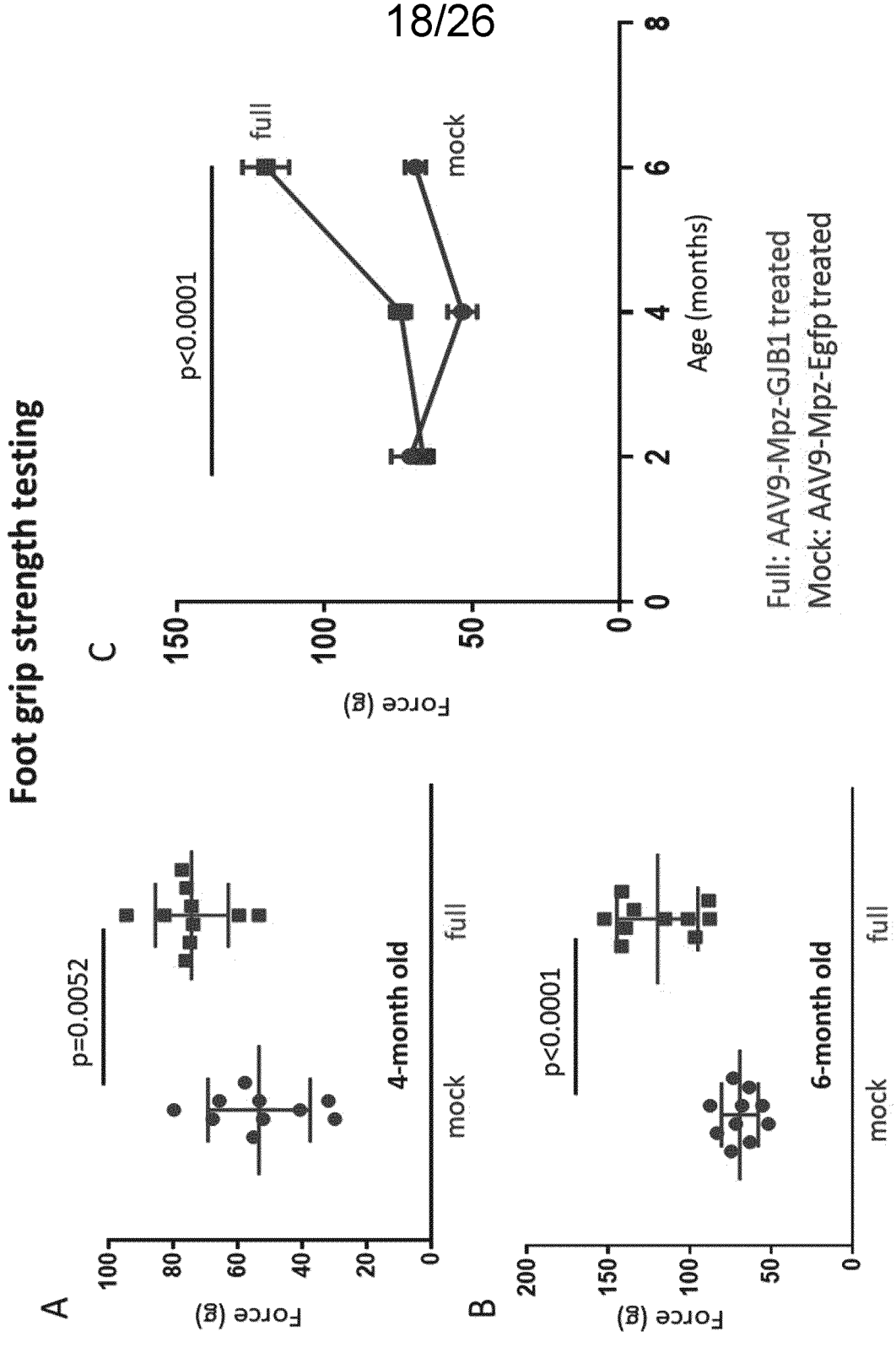

Foot grip strength testing
A
p=0.0052
Force (g)
100
80
60
40
20
0
4-month old
mock
full
B
p<0.0001
Force (g)
200
150
100
50
0
6-month old
mock
full
C
p<0.0001
Force (g)
150
100
50
full
mock
0
2
4
6
8
Age (months)
Full: AAV9-Mpz-GJB1 treated
Mock: AAV9-Mpz-Egfp treated

Figure 12

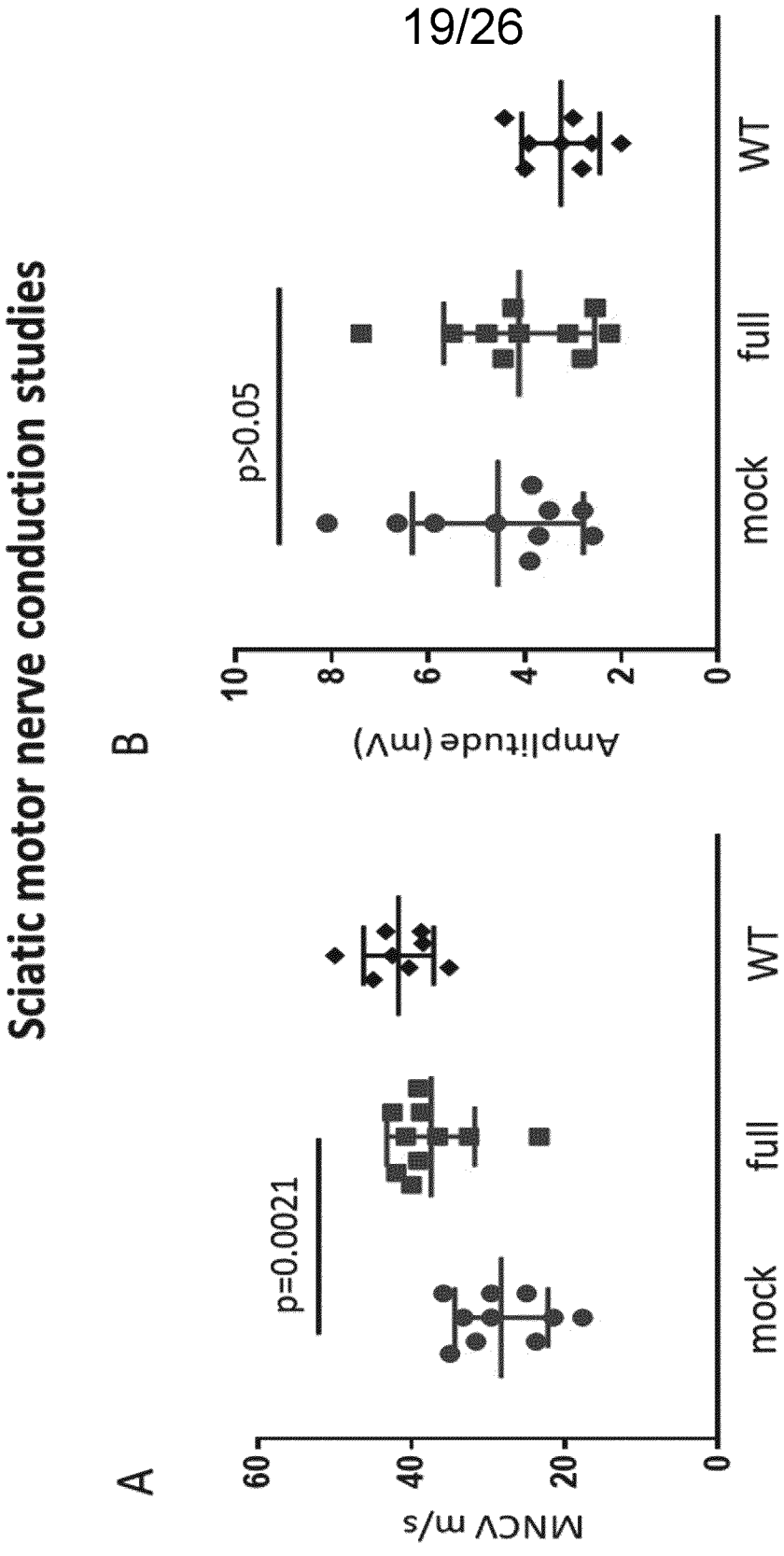

Sciatic motor nerve conduction studies
A
MNCV m/s
60
40
20
0
p=0.0021
mock
full
WT
B
Amplitude (mV)
10
8
6
4
2
0
p>0.05
mock
full
WT

Figure 13

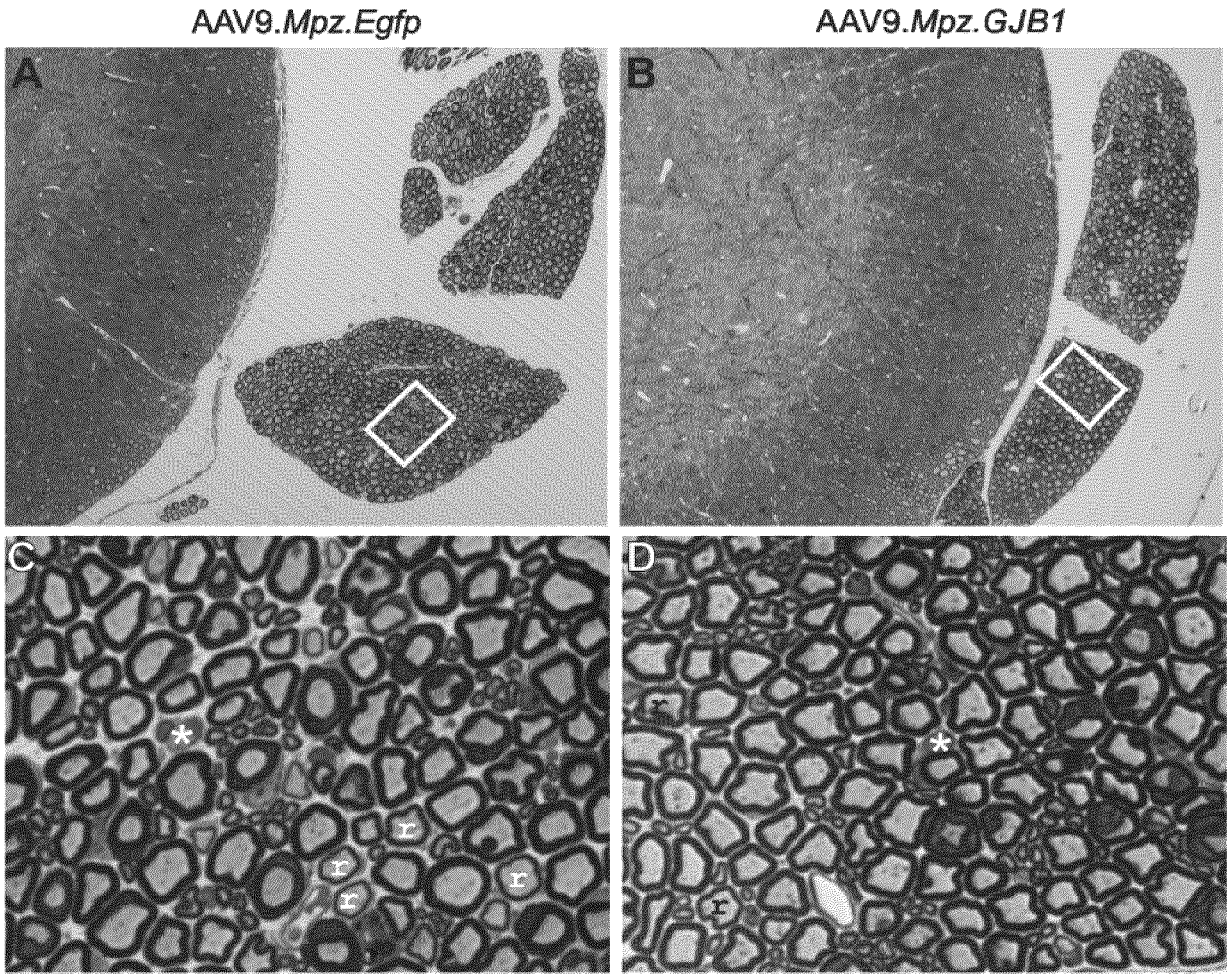
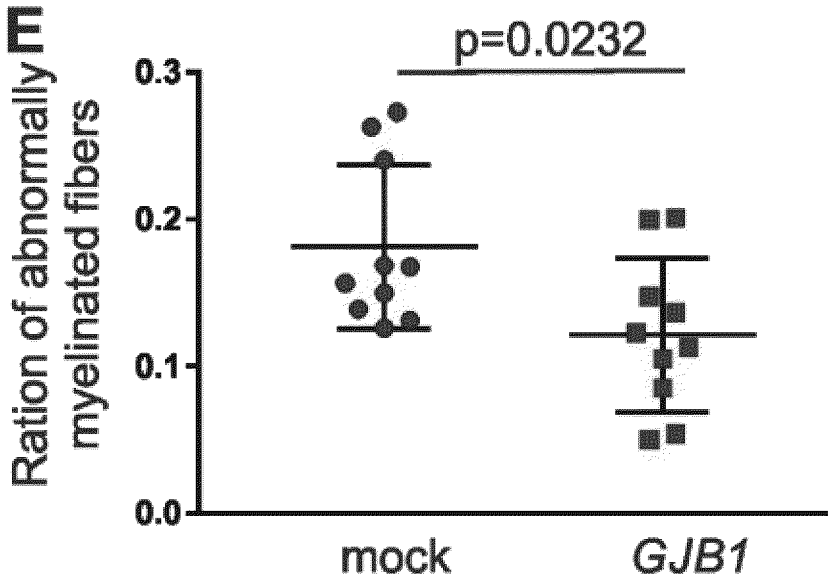



Figure 14
(page 1 of 2)



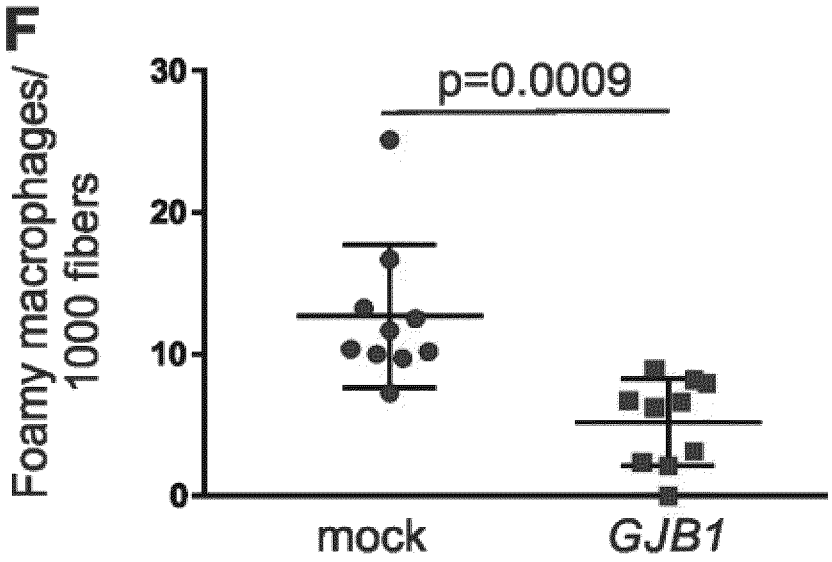




Figure 14
(page 2 of 2)

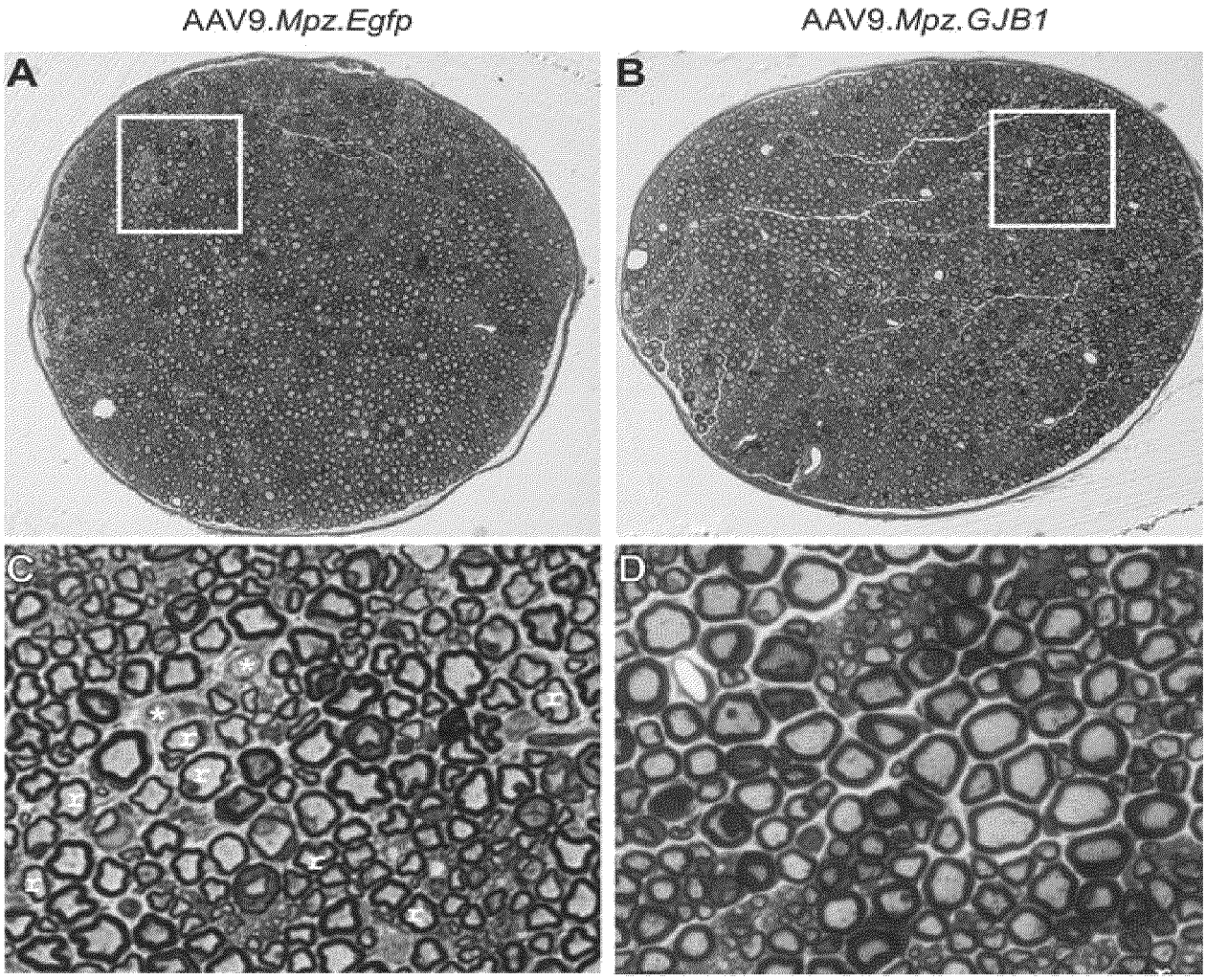



Figure 15
(part 1 of 2)

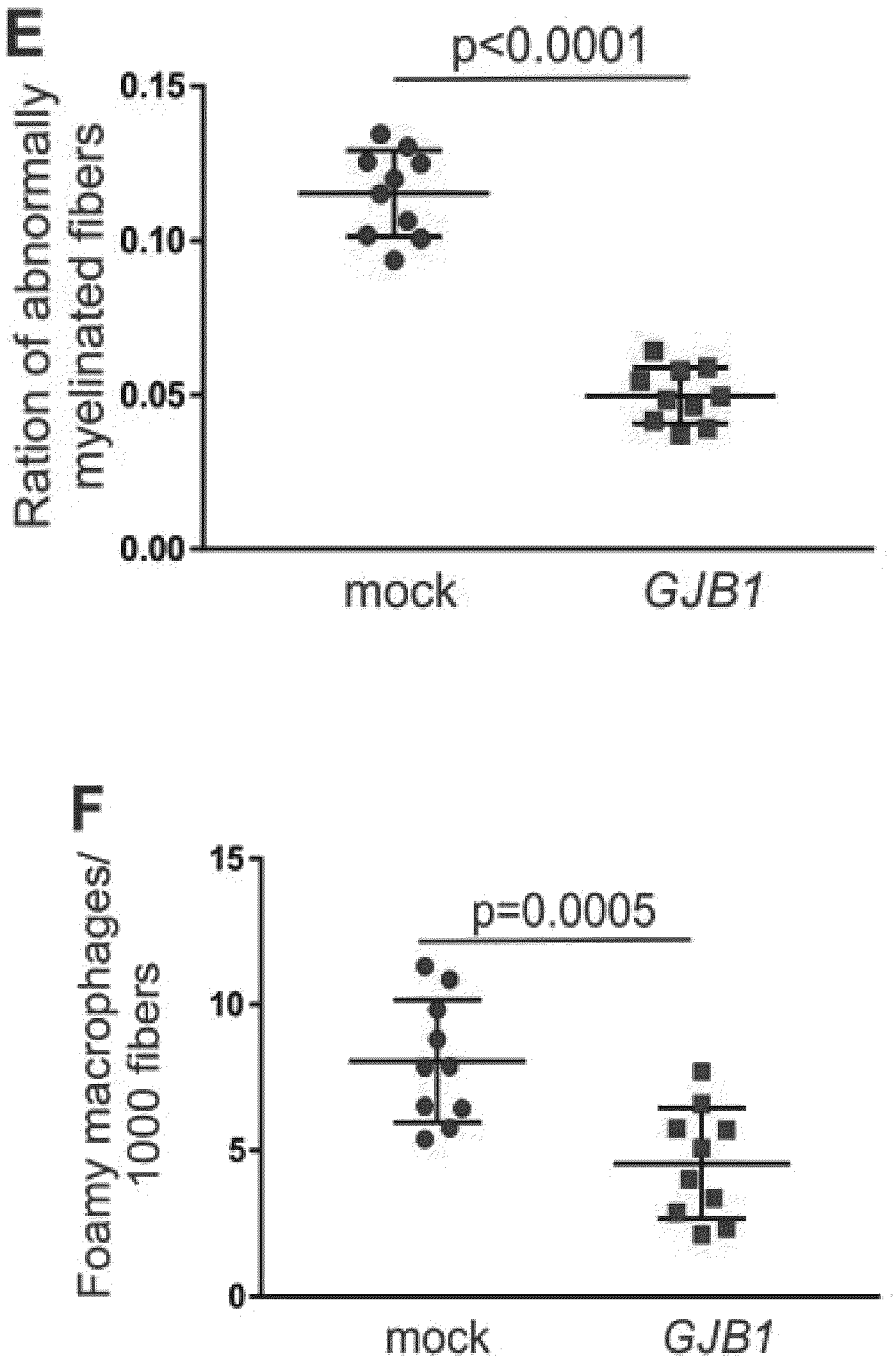



Figure 15
(part 2 of 2)

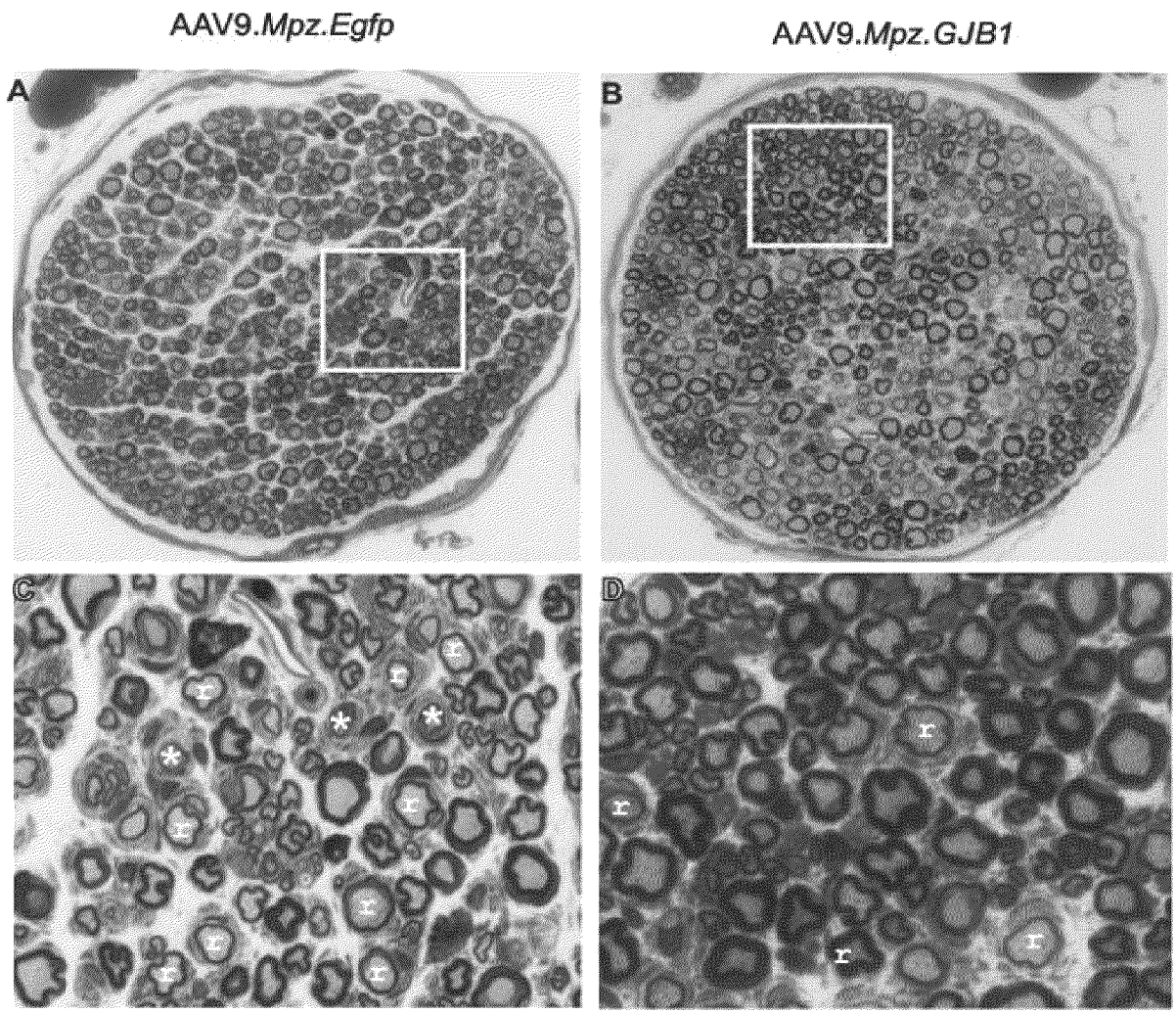
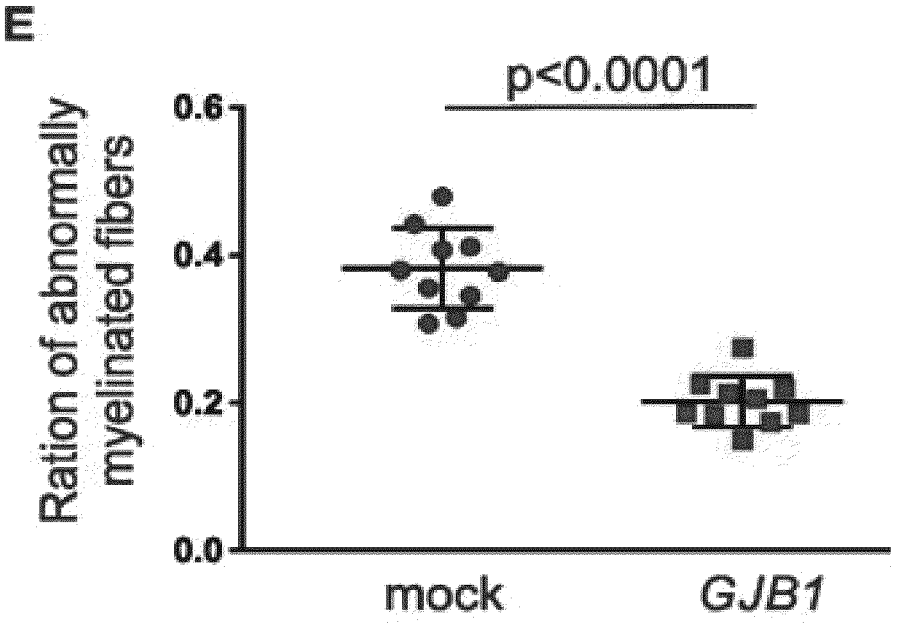



Figure 16
(part 1 of 2)



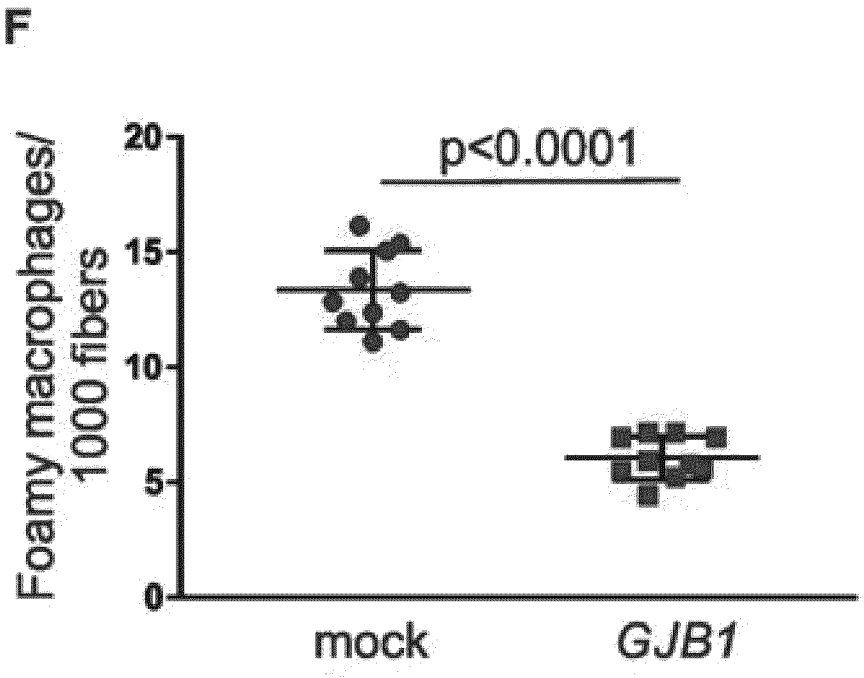
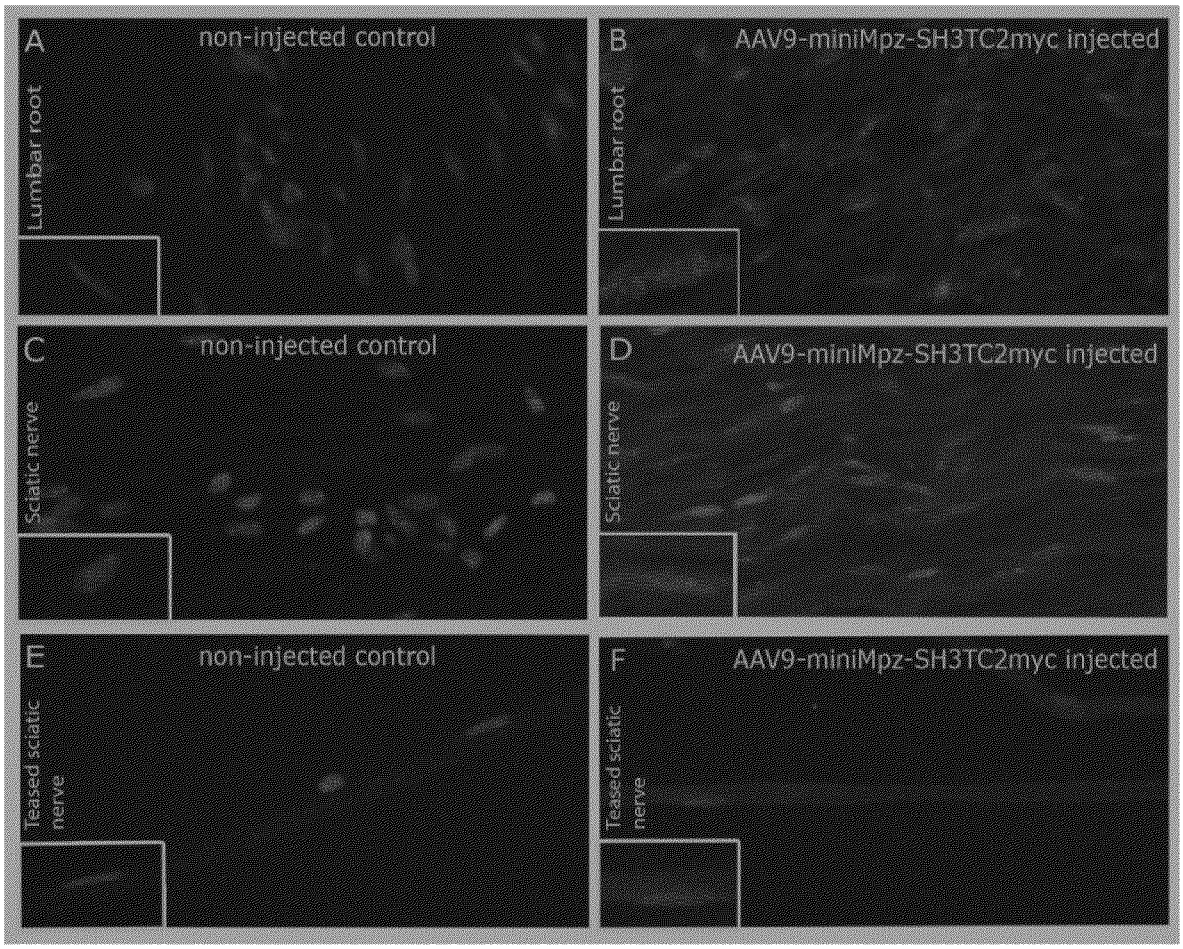




Figure 16
(part 2 of 2)


A
non-injected control
Lumbar root
B
AAV9-miniMpz-SH3TC2myc injected
Lumbar root
C
non-injected control
Sciatic nerve
D
AAV9-miniMpz-SH3TC2myc injected
Sciatic nerve
E
non-injected control
Teased sciatic nerve
F
AAV9-miniMpz-SH3TC2myc injected
Teased sciatic nerve

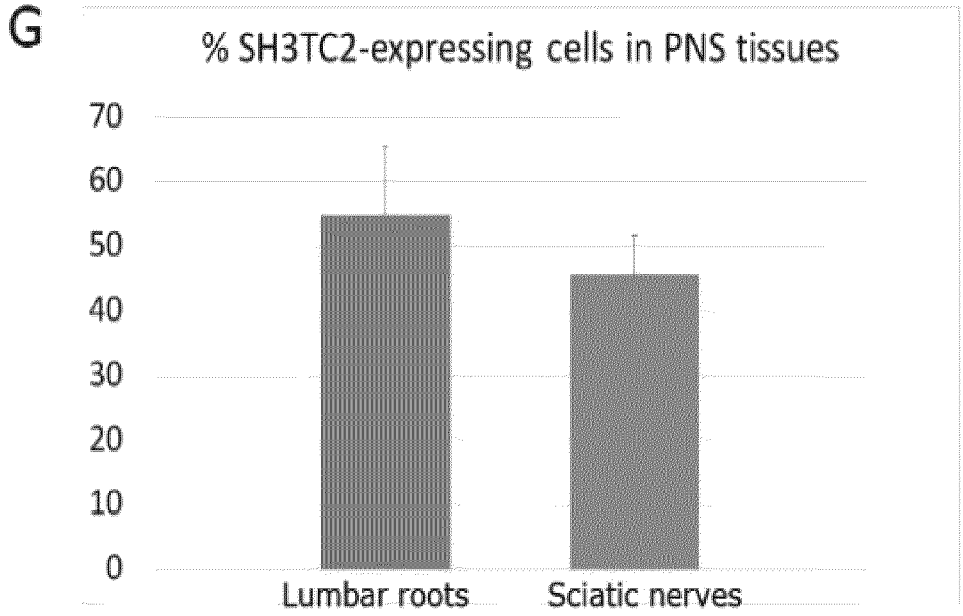

G
% SH3TC2-expressing cells in PNS tissues
70
60
50
40
30
20
10
0
Lumbar roots
Sciatic nerves

Figure 17