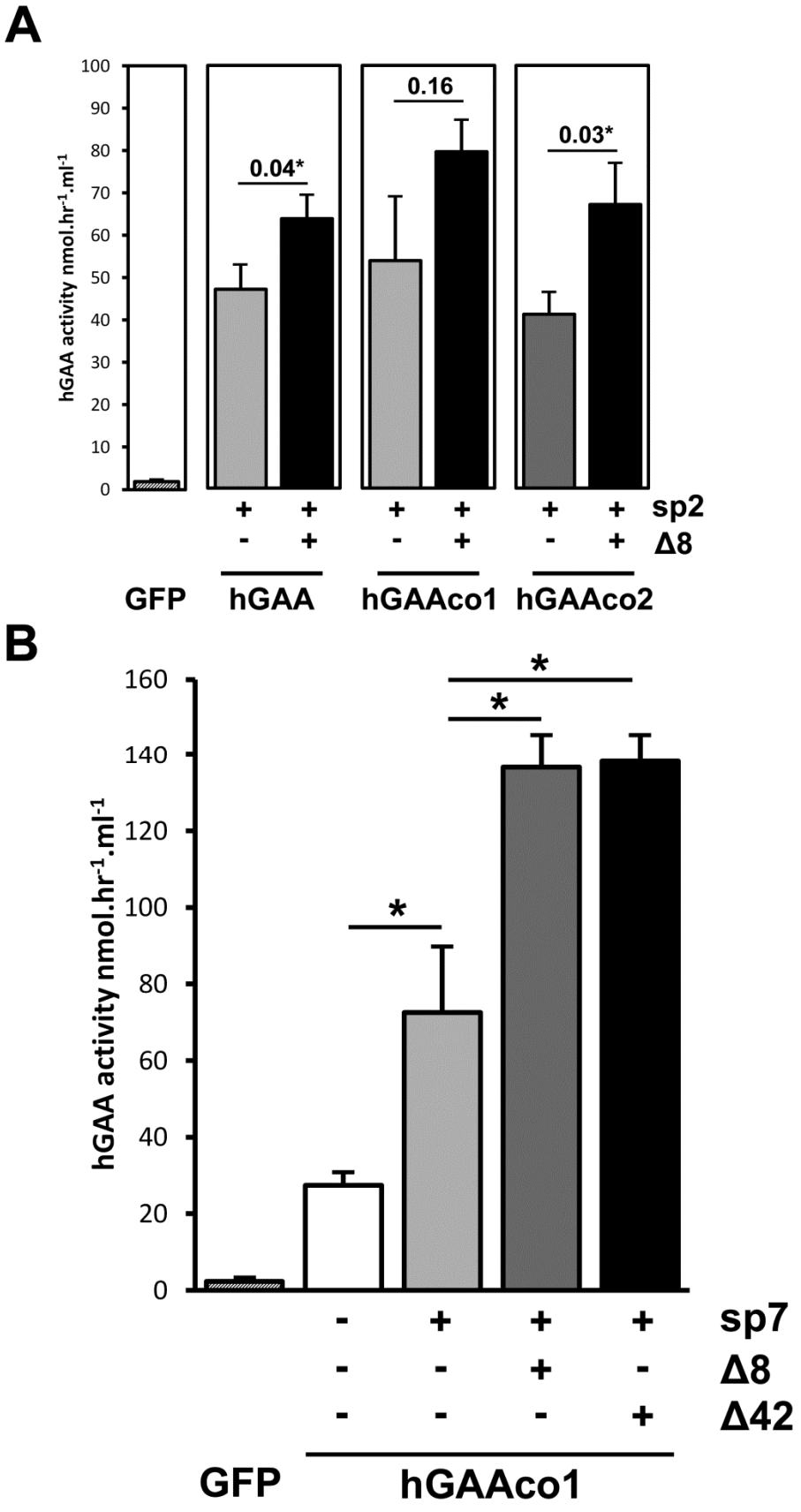
A
hGAA activity nmol.hr-1.ml-1
100
90
80
70
60
50
40
30
20
10
0
0.04*
0.16
0.03*
+ + + + + + sp2
- + - + - + Δ8
GFP
hGAA
hGAAco1
hGAAco2
B
hGAA activity nmol.hr-1.ml-1
160
140
120
100
80
60
40
20
0
*
*
*
- + + + sp7
- - + - Δ8
- - - + Δ42
GFP
hGAAco1

FIGURE 1

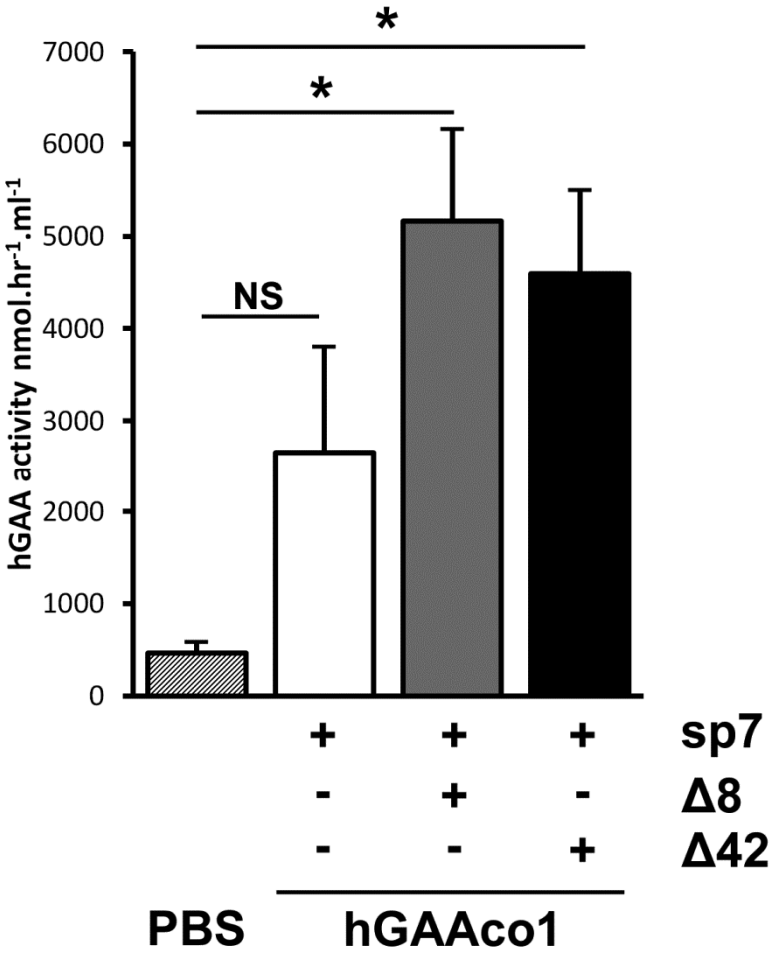
*
*
7000
6000
5000
4000
3000
2000
1000
0
hGAA activity nmol.hr-1.ml-1
NS
+ + + sp7
- + - Δ8
- - + Δ42
PBS
hGAAco1

FIGURE 2

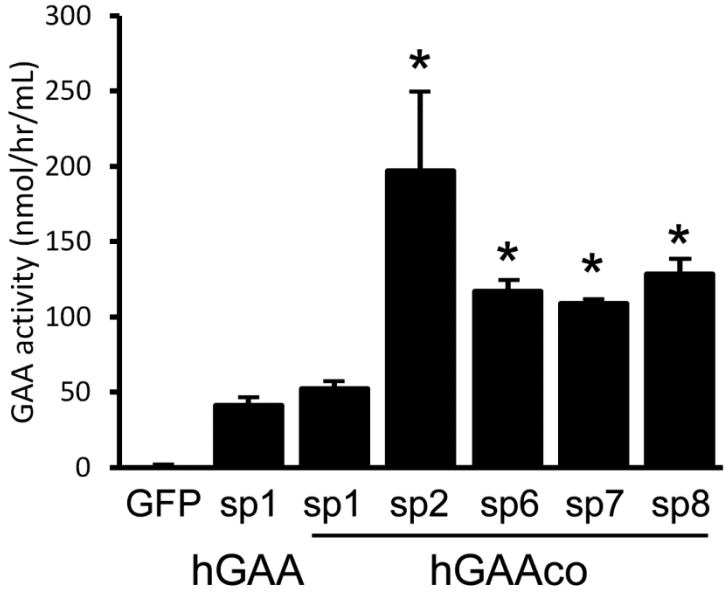
300
250
200
150
100
50
0
GAA activity (nmol/hr/mL)
*
*
*
*
GFP
sp1
sp1
sp2
sp6
sp7
sp8
hGAA
hGAAco

FIGURE 3

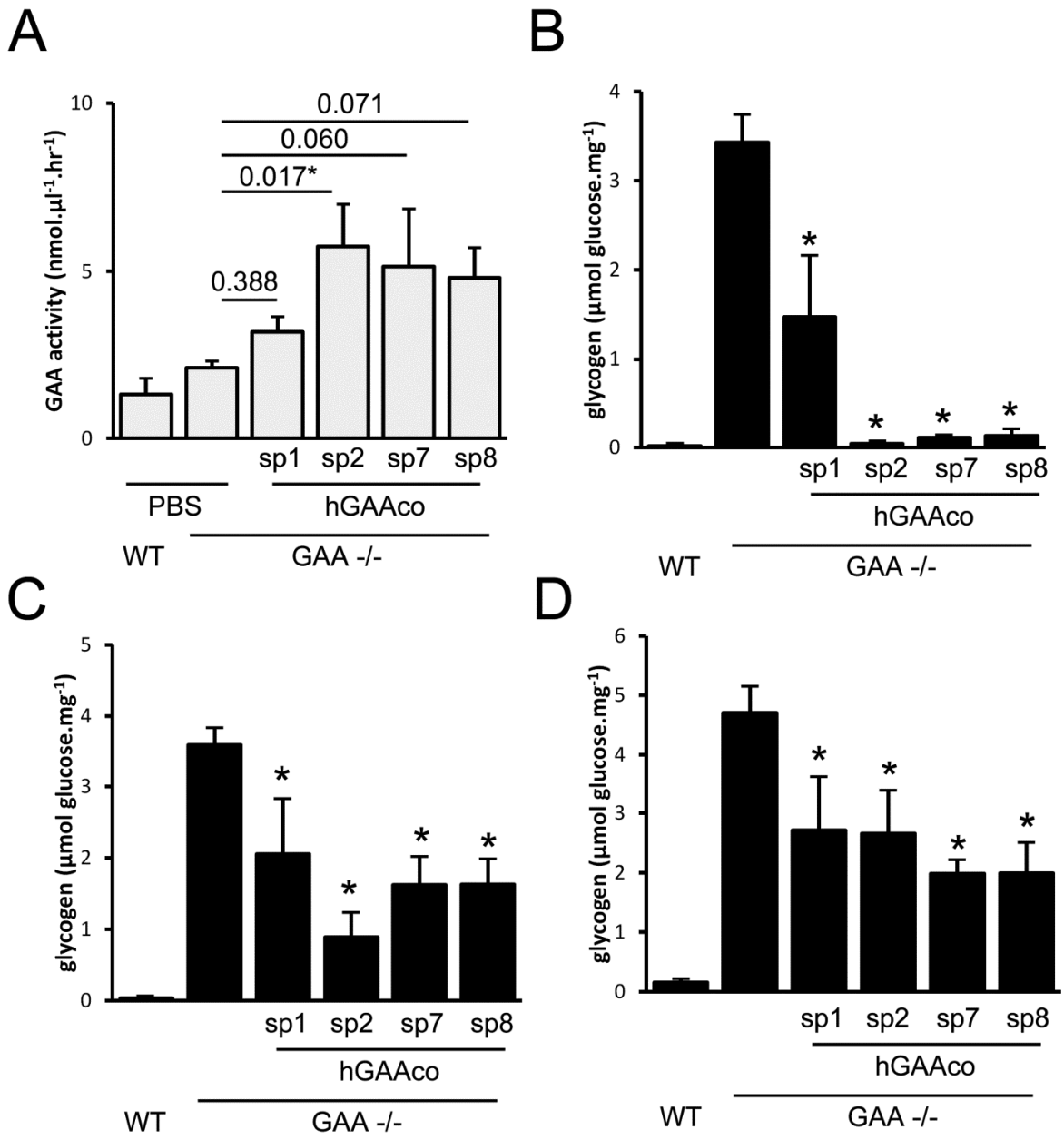
A
B
C
D
GAA activity (nmol.µl-1.hr-1)
glycogen (µmol glucose.mg-1)
0.071
0.060
0.017*
0.388
sp1
sp2
sp7
sp8
PBS
hGAAco
WT
GAA -/-

FIGURE 4

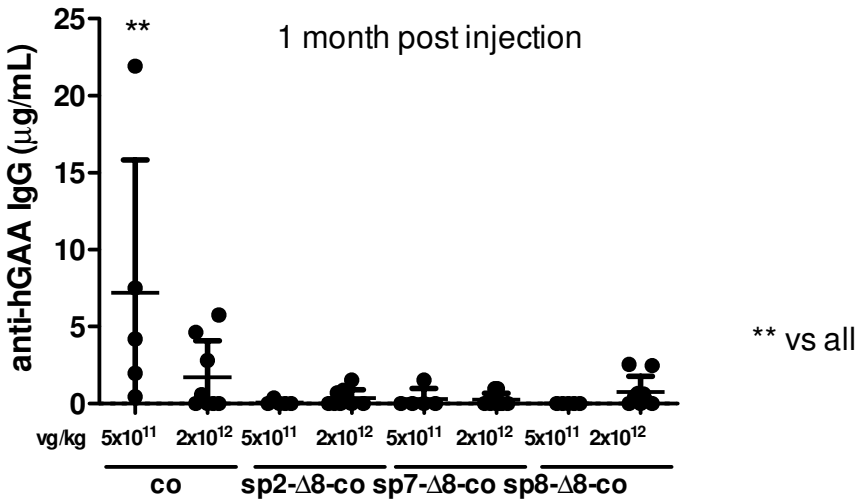
1 month post injection
anti-hGAA IgG (µg/mL)
25
20
15
10
5
0
**
** vs all
vg/kg
5x10^11
2x10^12
5x10^11
2x10^12
5x10^11
2x10^12
5x10^11
2x10^12
co
sp2-Δ8-co
sp7-Δ8-co
sp8-Δ8-co

FIGURE 5

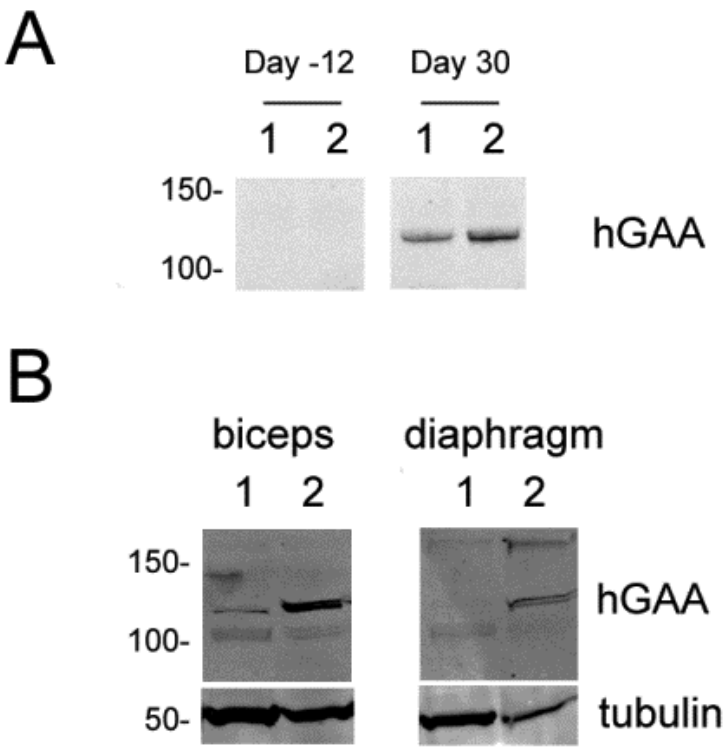
A
Day -12
Day 30
1 2 1 2
150-
100-
hGAA
B
biceps
diaphragm
1 2 1 2
150-
100-
50-
hGAA
tubulin

FIGURE 6

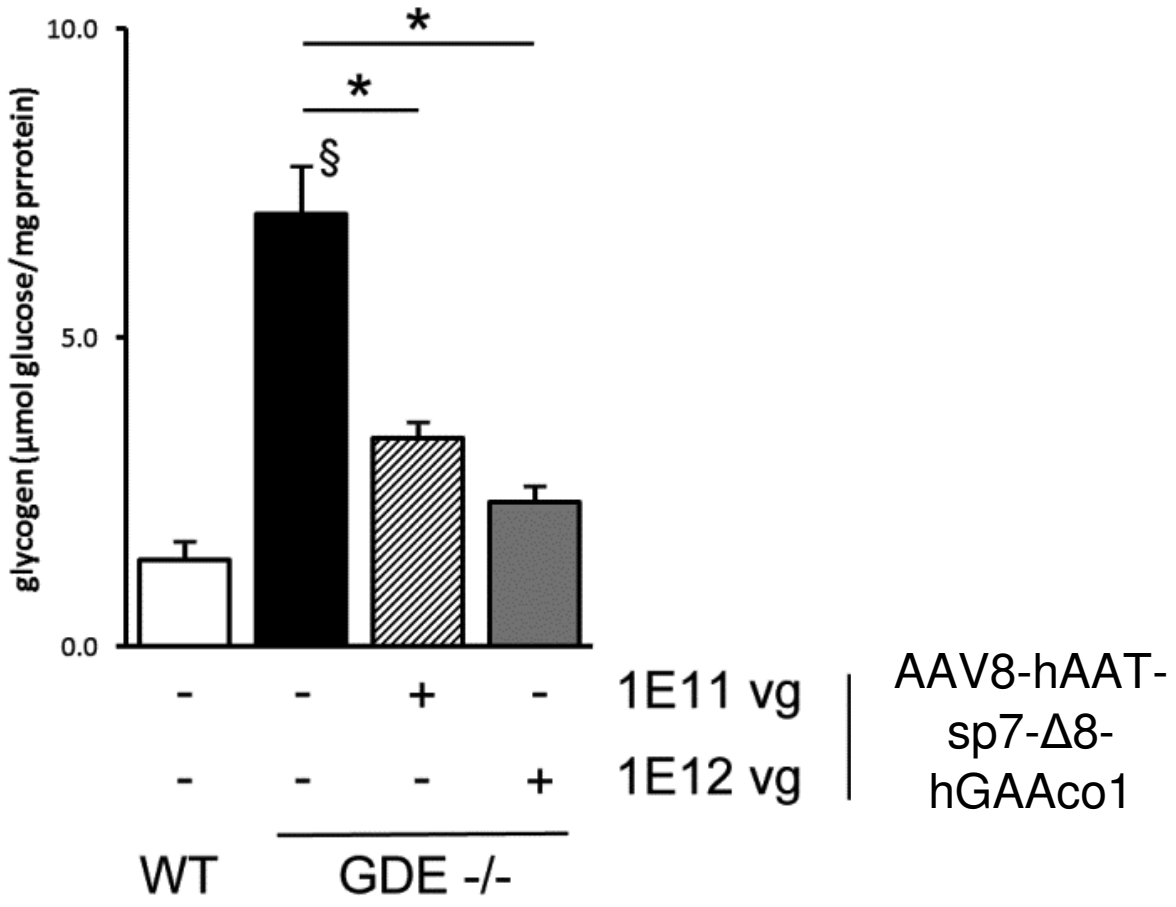
10.0
5.0
0.0
glycogen (µmol glucose/mg prrotein)
*
*
§
- - + - 1E11 vg
- - - + 1E12 vg
AAV8-hAAT-sp7-Δ8-hGAAco1
WT
GDE -/-

FIGURE 7

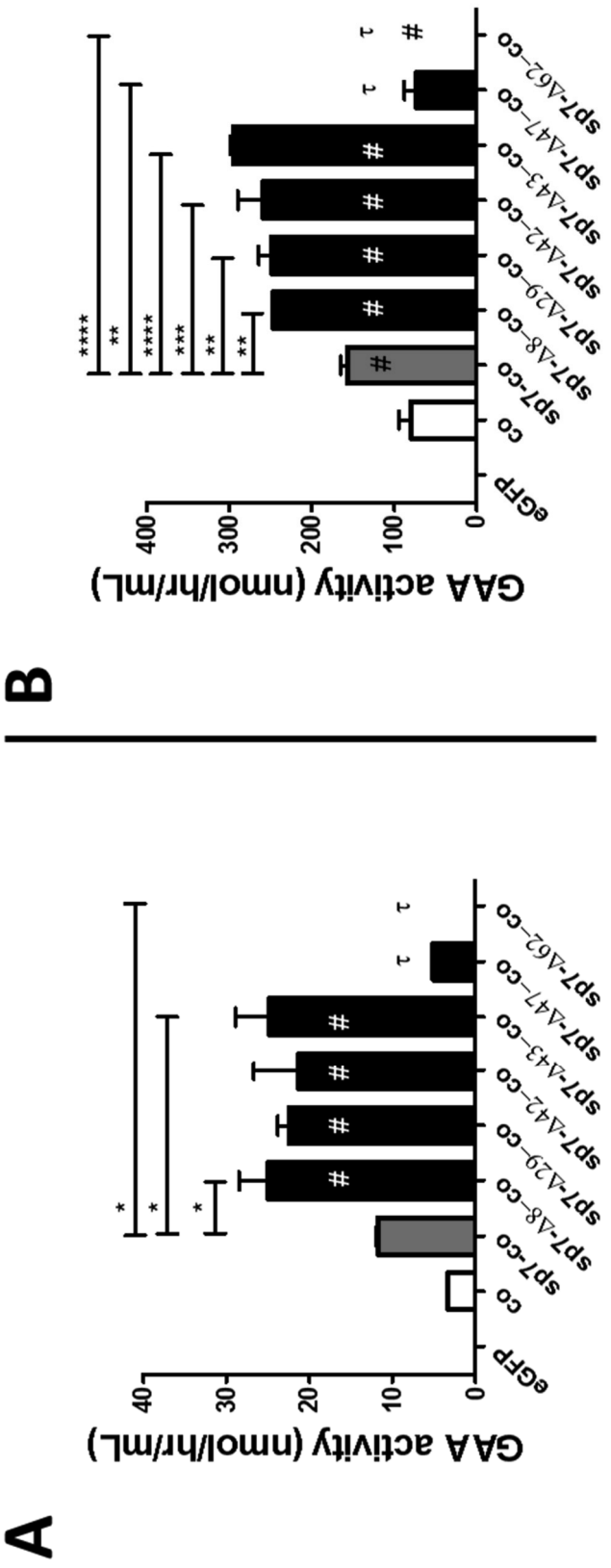
A
GAA activity (nmol/hr/mL)
0
10
20
30
40
eGFP
co
spT-co
spT-A8-co
spT-A29-co
spT-A42-co
spT-A43-co
spT-A47-co
spT-A62-co
B
GAA activity (nmol/hr/mL)
0
100
200
300
400
eGFP
co
spT-co
spT-A8-co
spT-A29-co
spT-A42-co
spT-A43-co
spT-A47-co
spT-A62-co

FIGURE 8

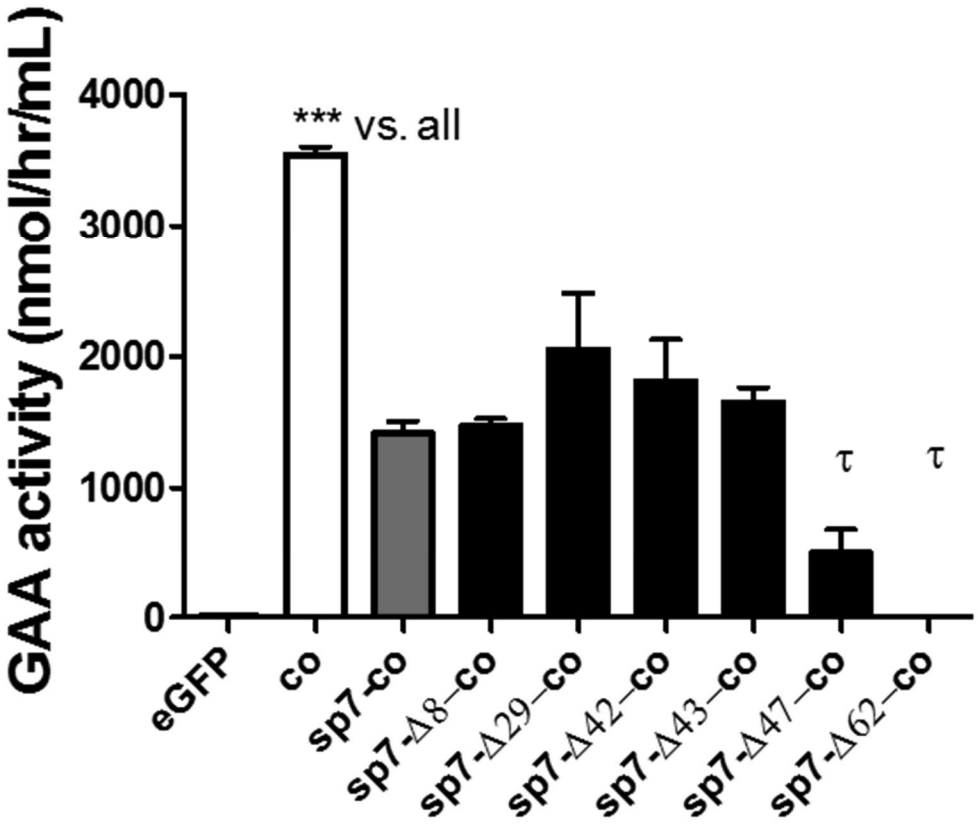
GAA activity (nmol/hr/mL)
4000
3000
2000
1000
0
*** vs. all
τ
τ
eGFP
co
sp7-co
sp7-Δ8-co
sp7-Δ29-co
sp7-Δ42-co
sp7-Δ43-co
sp7-Δ47-co
sp7-Δ62-co

FIGURE 9

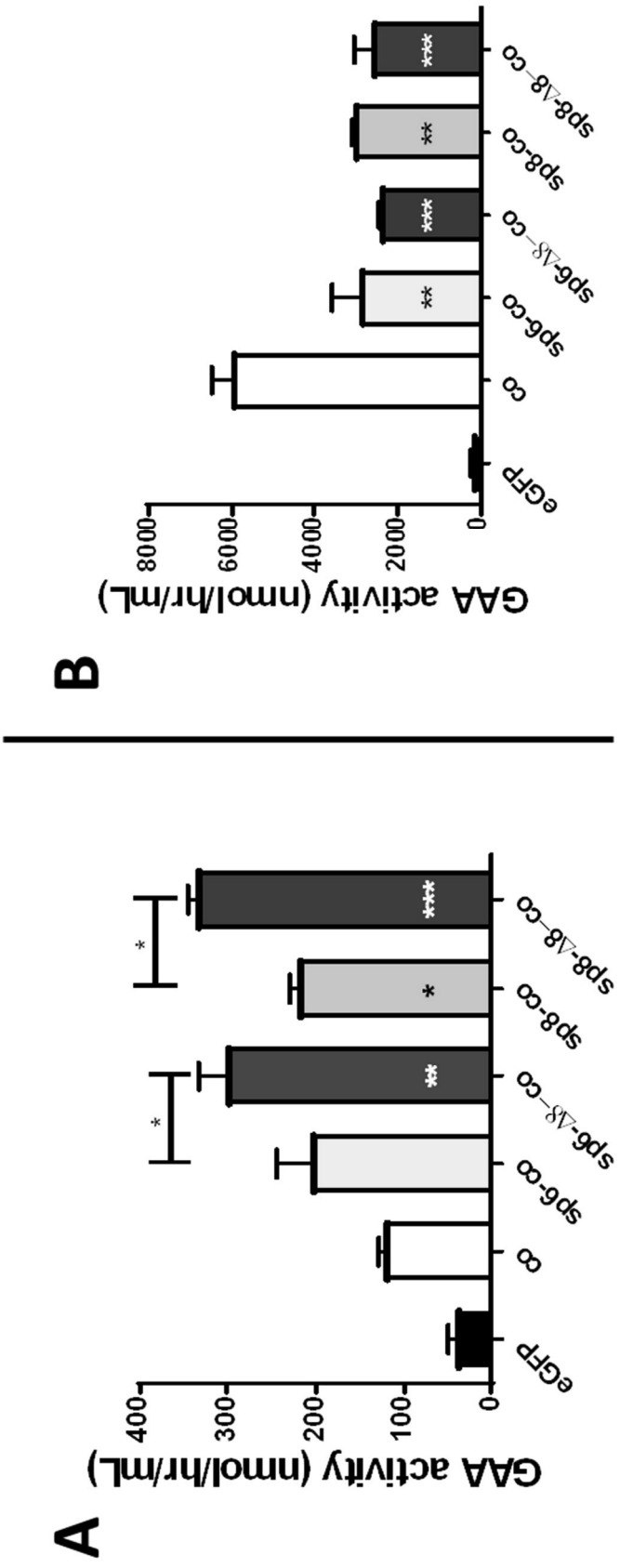
A
GAA activity (nmol/hr/mL)
0
100
200
300
400
eGFP
co
spB-co
spB-Δ8-co
spB-Δ8-co
spB-co
co
eGFP
GAA activity (nmol/hr/mL)
0
2000
4000
6000
8000
B

FIGURE 10