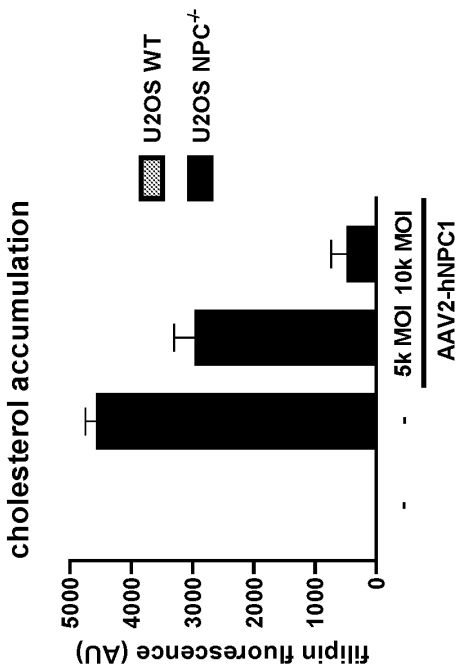

cholesterol accumulation
U2OS WT
U2OS NPC-/-
filipin fluorescence (AU)
5000
4000
3000
2000
1000
0
-
-
5k MOI 10k MOI
AAV2-hNPC1

FIG. 1B

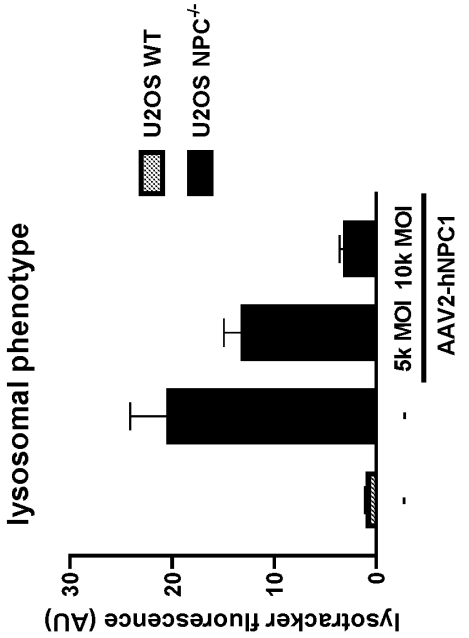

lysosomal phenotype
U2OS WT
U2OS NPC-/-
lysotracker fluorescence (AU)
30
20
10
0
-
-
5k MOI 10k MOI
AAV2-hNPC1

FIG. 1A

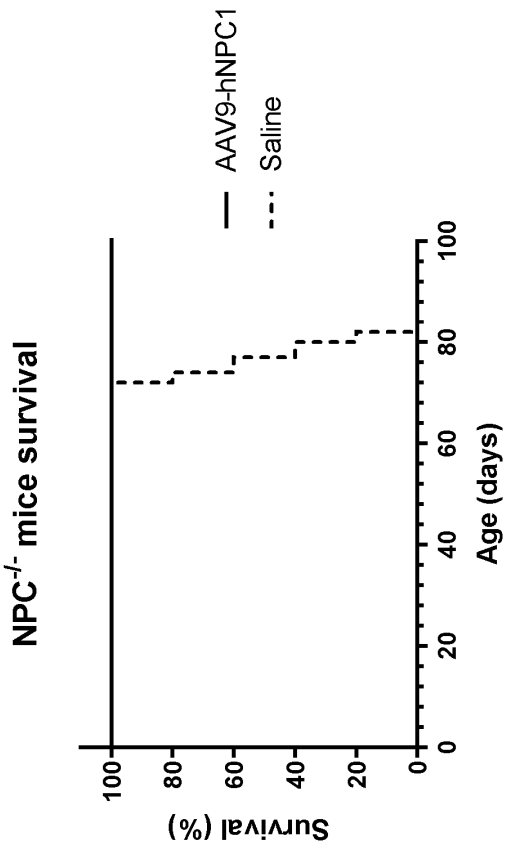

NPC-/- mice survival
AAV9-hNPC1
Saline
Survival (%)
100
80
60
40
20
0
0
20
40
60
80
100
Age (days)

FIG. 2

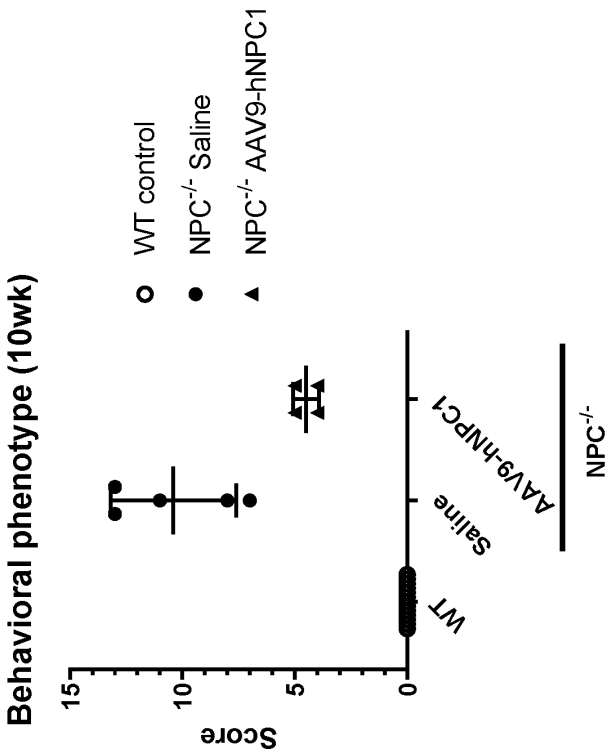

Behavioral phenotype (10wk)
WT control
NPC-/- Saline
NPC-/- AAV9-hNPC1
Score
15
10
5
0
WT
Saline
AAV9-hNPC1
NPC-/-

FIG. 3

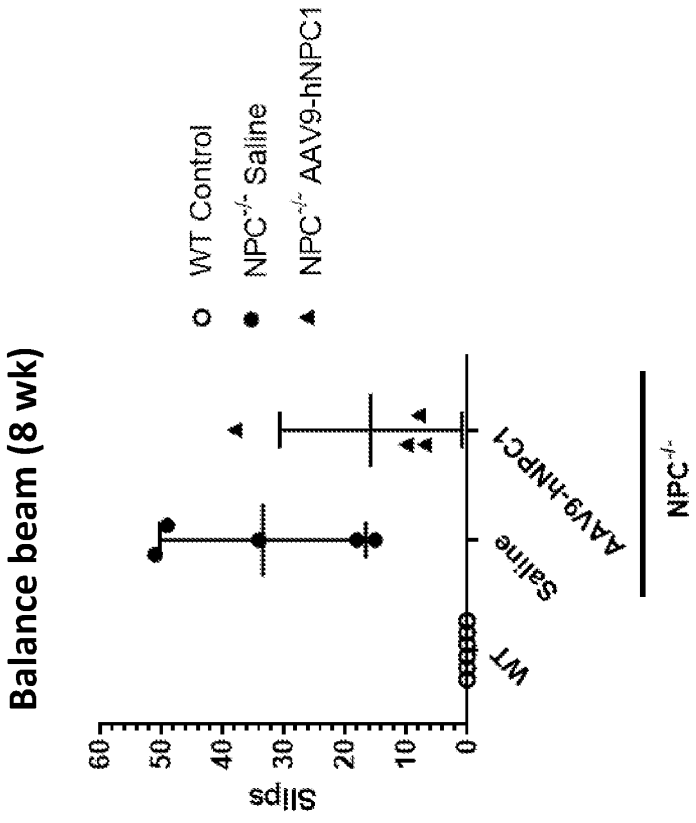

Balance beam (8 wk)
WT Control
NPC-/- Saline
NPC-/- AAV9-hNPC1
Slips
60
50
40
30
20
10
0
WT
Saline
AAV9-hNPC1
NPC-/-

FIG. 4