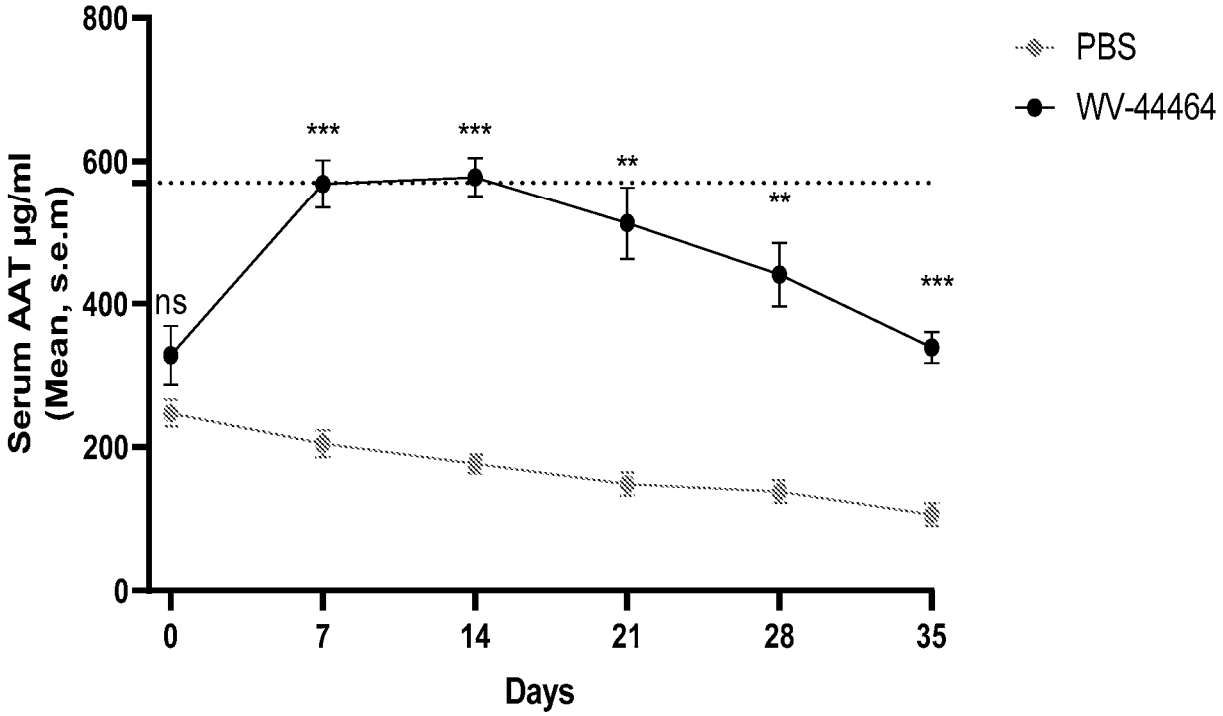
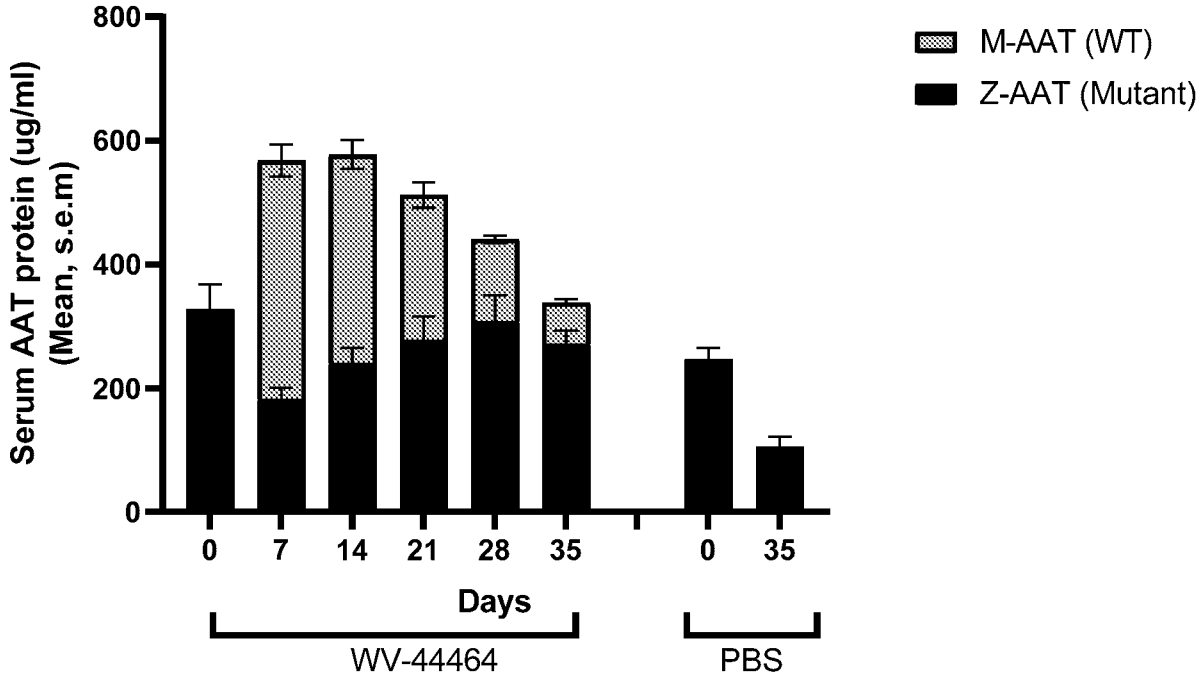

800
600
400
200
0
Serum AAT µg/ml
(Mean, s.e.m)
ns
***
***
**
**
***
0
7
14
21
28
35
Days
PBS
WV-44464

(a)


800
600
400
200
0
Serum AAT protein (ug/ml)
(Mean, s.e.m)
0
7
14
21
28
35
0
35
Days
WV-44464
PBS
M-AAT (WT)
Z-AAT (Mutant)

(b)

FIG. 1

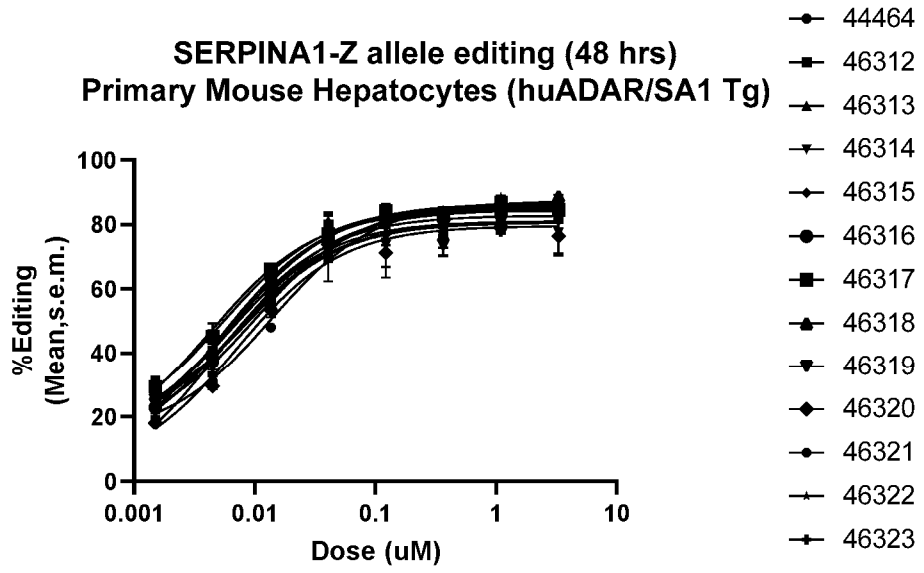


FIG. 2

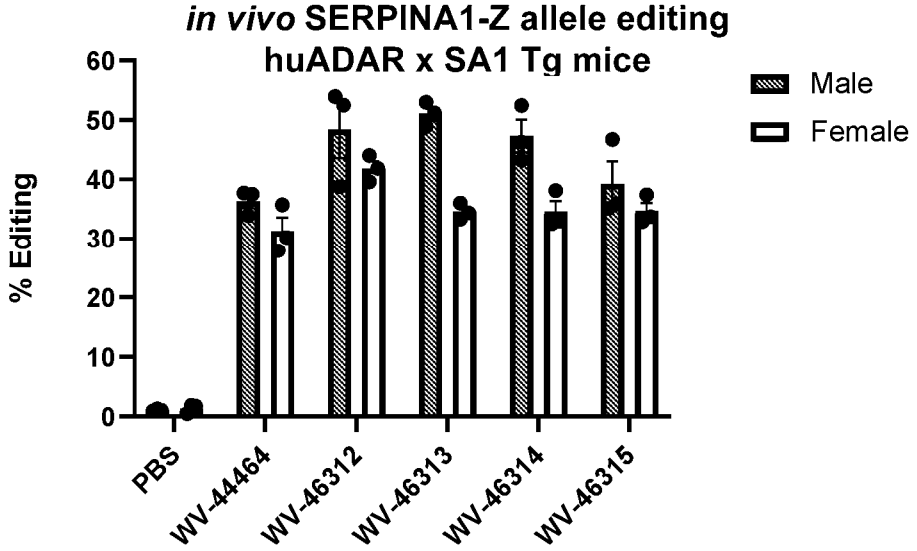


FIG. 3

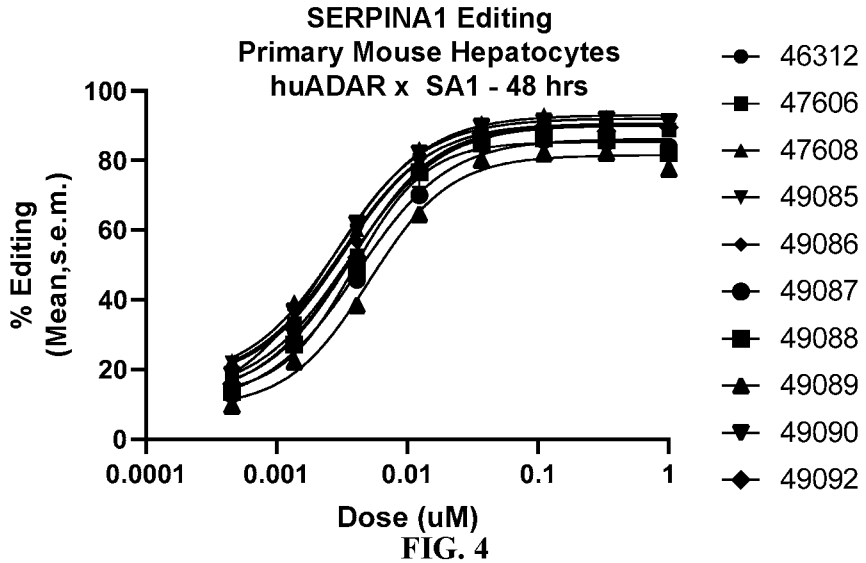


FIG. 4

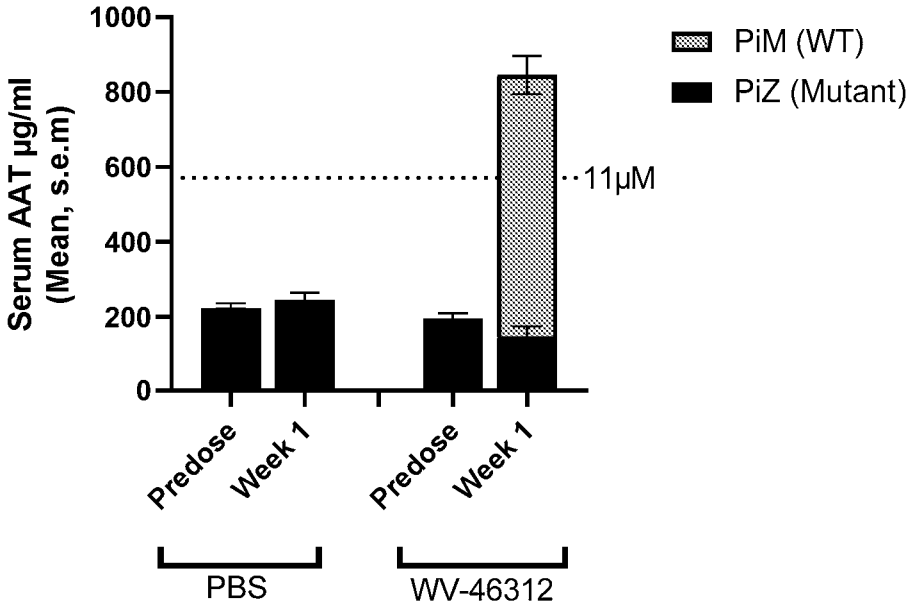


FIG. 5

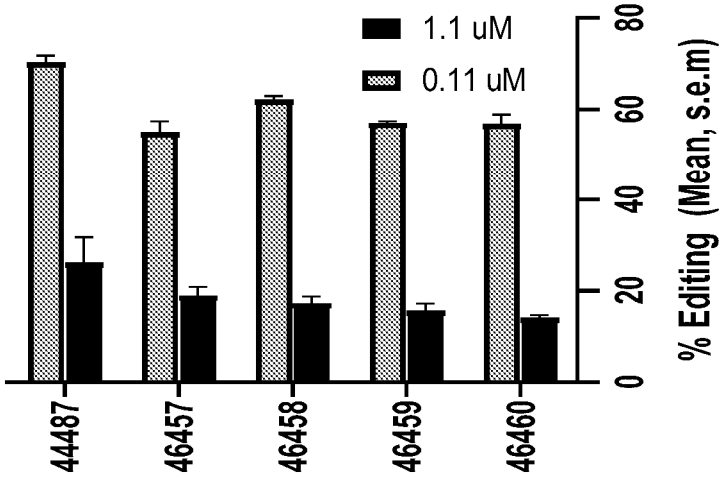


FIG. 6

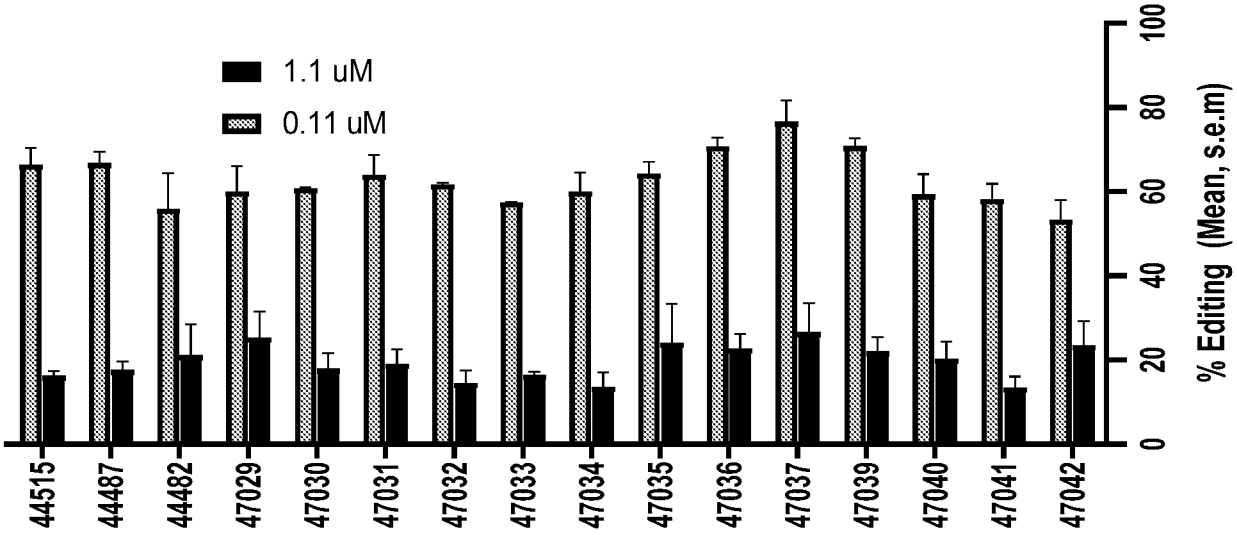


FIG. 7

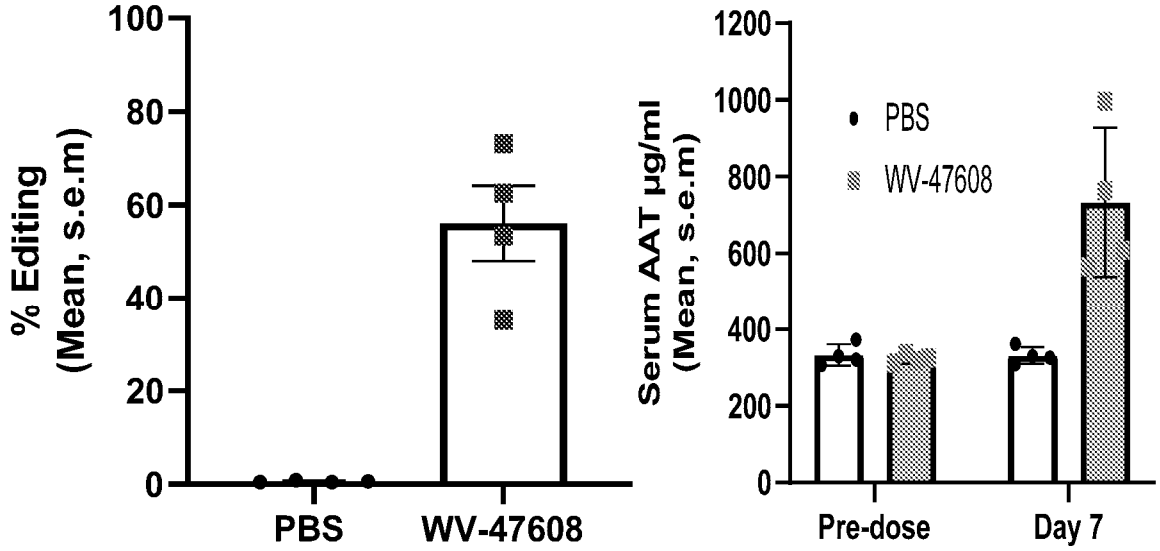

% Editing
(Mean, s.e.m)
100
80
60
40
20
0
PBS
WV-47608
Serum AAT µg/ml
(Mean, s.e.m)
1200
1000
800
600
400
200
0
PBS
WV-47608
Pre-dose
Day 7

FIG. 8

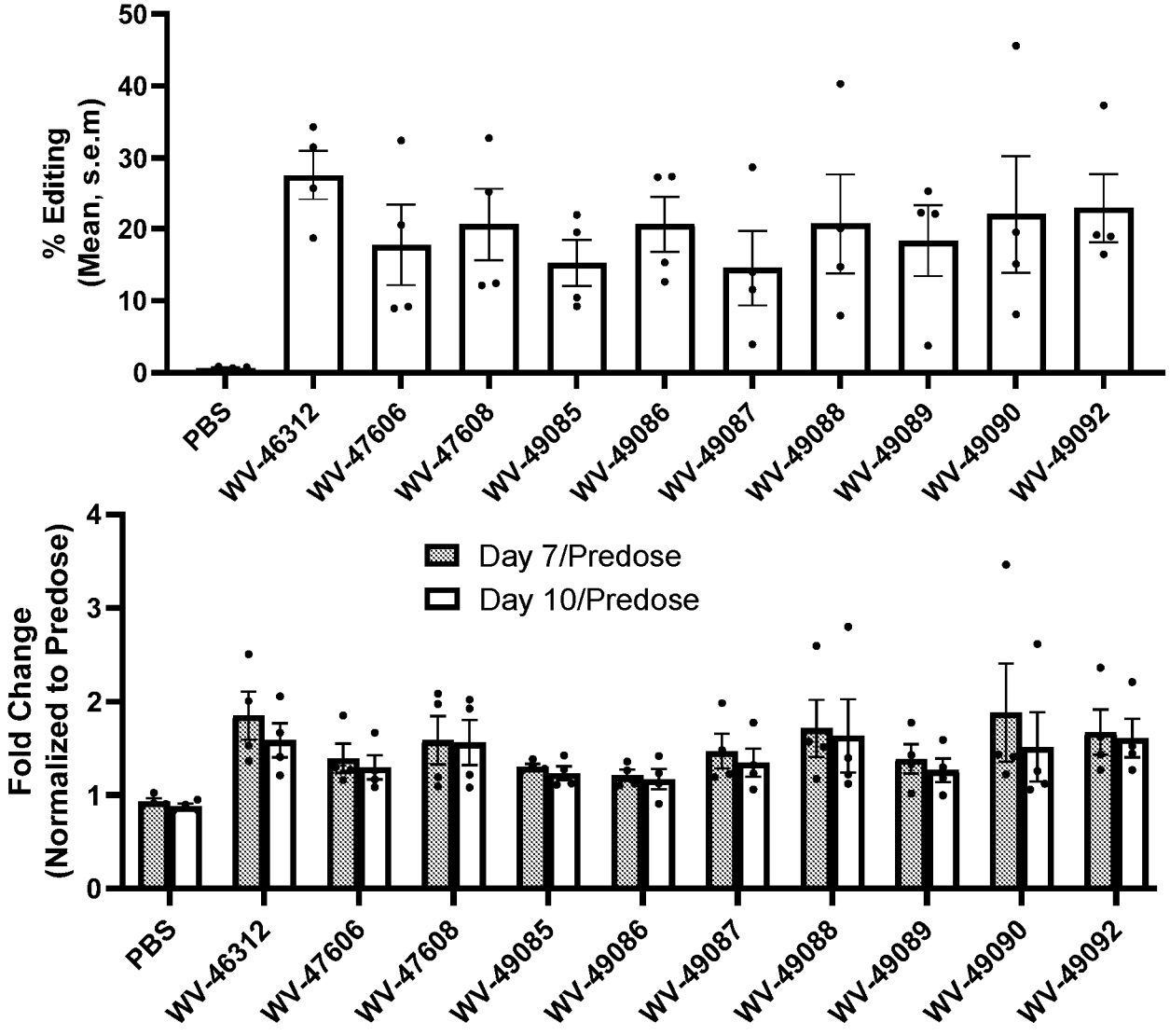

% Editing
(Mean, s.e.m)
50
40
30
20
10
0
PBS
WV-46312
WV-47606
WV-47608
WV-49085
WV-49086
WV-49087
WV-49088
WV-49089
WV-49090
WV-49092
Fold Change
(Normalized to Predose)
4
3
2
1
0
Day 7/Predose
Day 10/Predose
PBS
WV-46312
WV-47606
WV-47608
WV-49085
WV-49086
WV-49087
WV-49088
WV-49089
WV-49090
WV-49092

FIG. 9

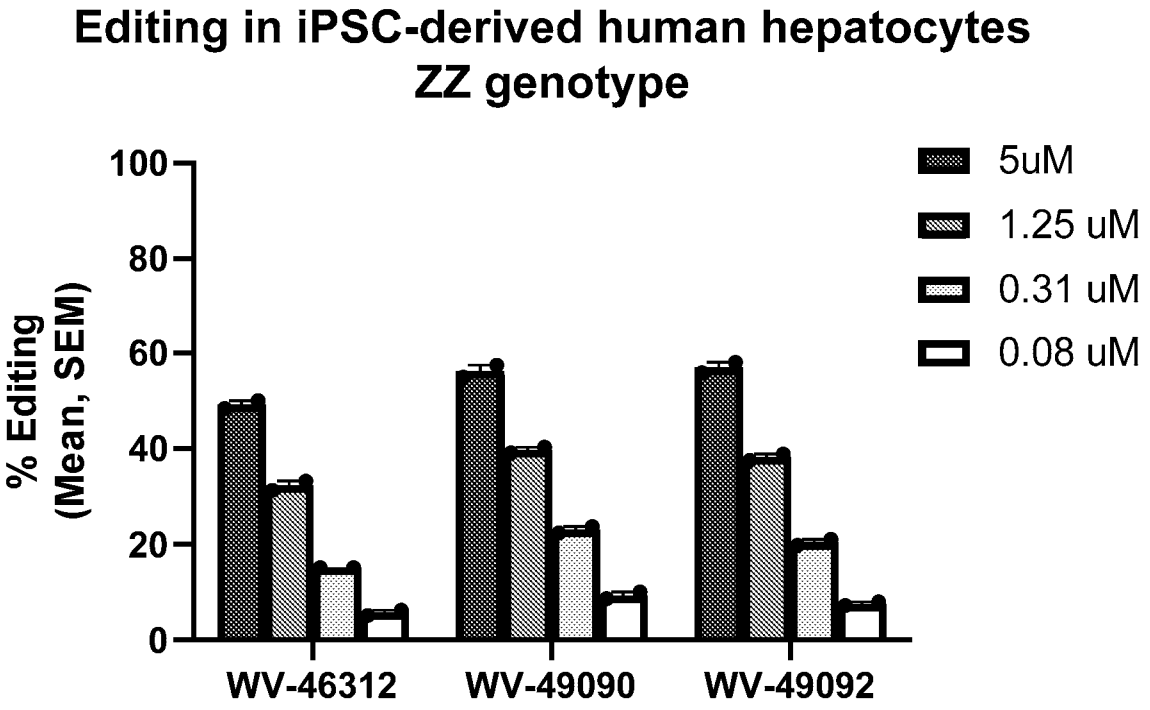

Editing in iPSC-derived human hepatocytes
ZZ genotype
% Editing
(Mean, SEM)
100
80
60
40
20
0
5uM
1.25 uM
0.31 uM
0.08 uM
WV-46312
WV-49090
WV-49092

FIG. 10

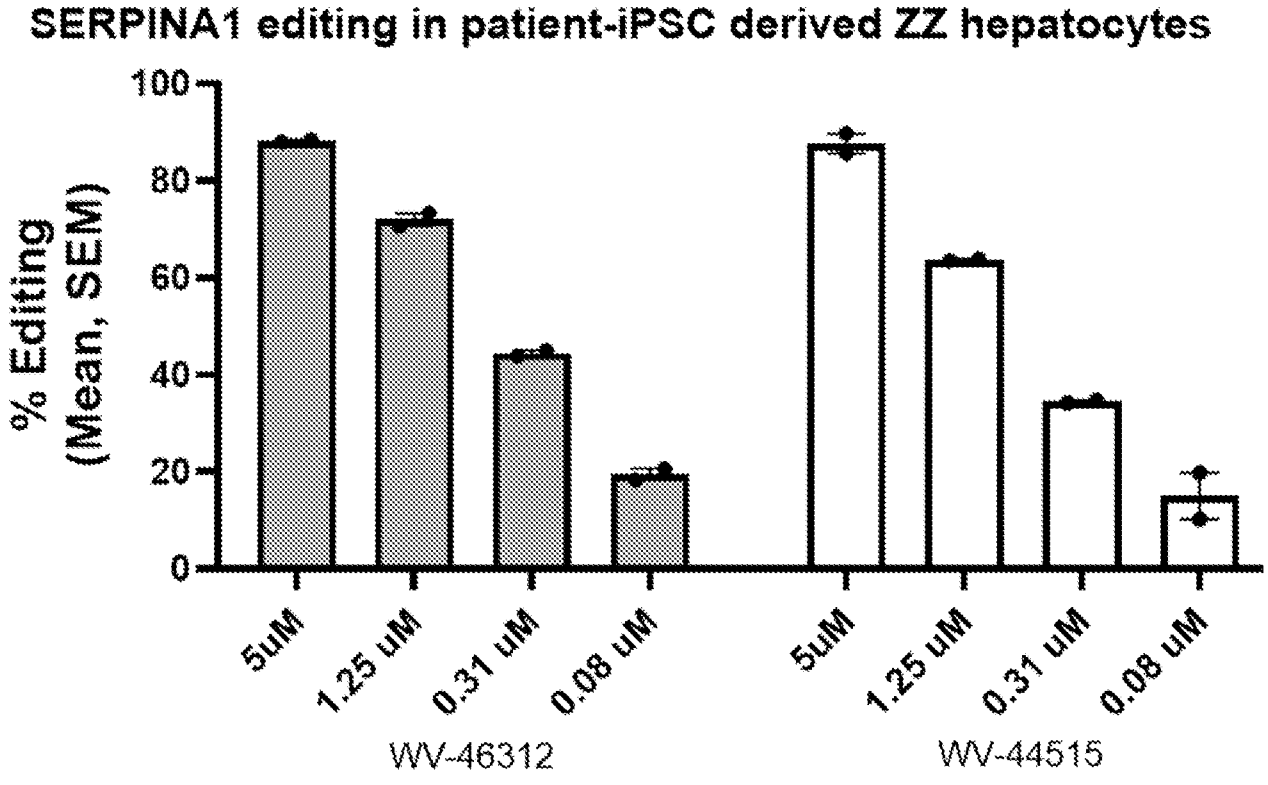

SERPINA1 editing in patient-iPSC derived ZZ hepatocytes
% Editing
(Mean, SEM)
100
80
60
40
20
0
5uM
1.25 uM
0.31 uM
0.08 uM
WV-46312
5uM
1.25 uM
0.31 uM
0.08 uM
WV-44515

FIG. 11

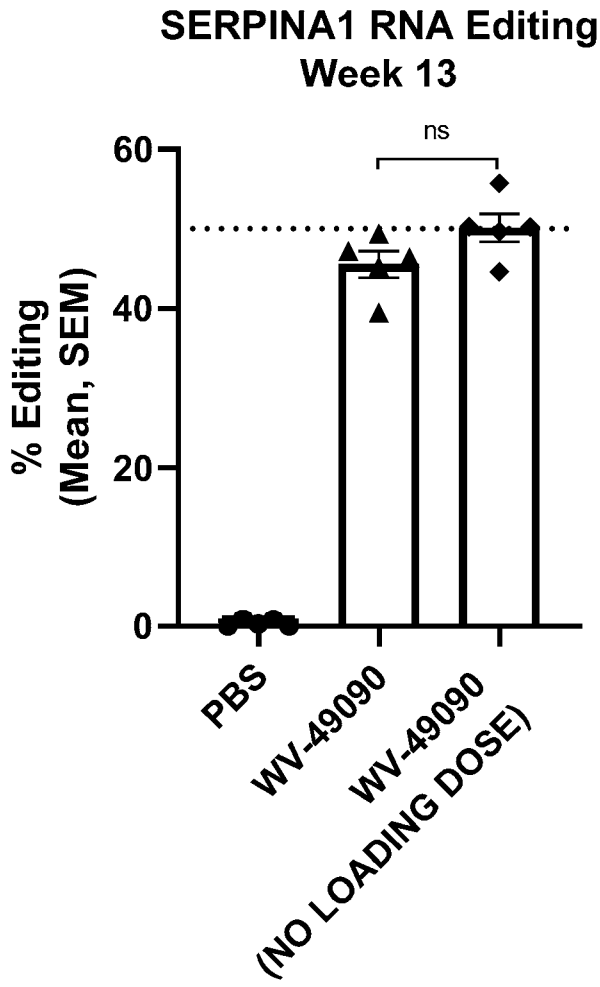

SERPINA1 RNA Editing
Week 13
ns
60
40
20
0
% Editing
(Mean, SEM)
PBS
WV-49090
WV-49090
(NO LOADING DOSE)

FIG. 12

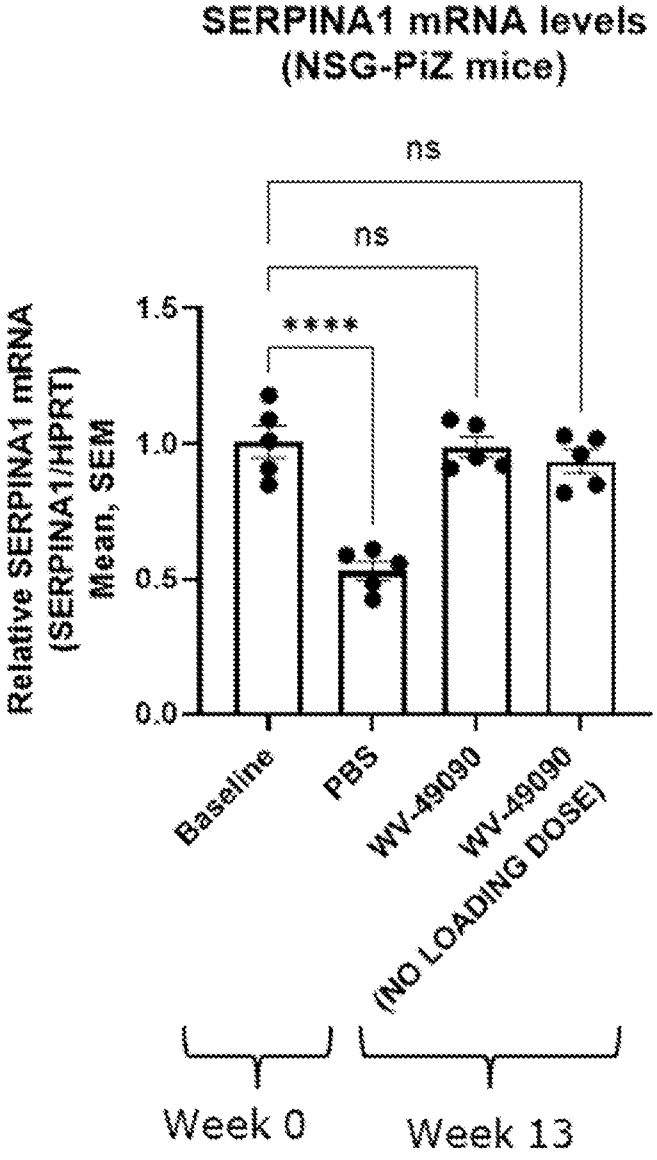

SERPINA1 mRNA levels
(NSG-PiZ mice)
ns
ns
****
Relative SERPINA1 mRNA
(SERPINA1/HPRT)
Mean, SEM
1.5
1.0
0.5
0.0
Baseline
PBS
WV-49090
WV-49090
(NO LOADING DOSE)
Week 0
Week 13

FIG. 13

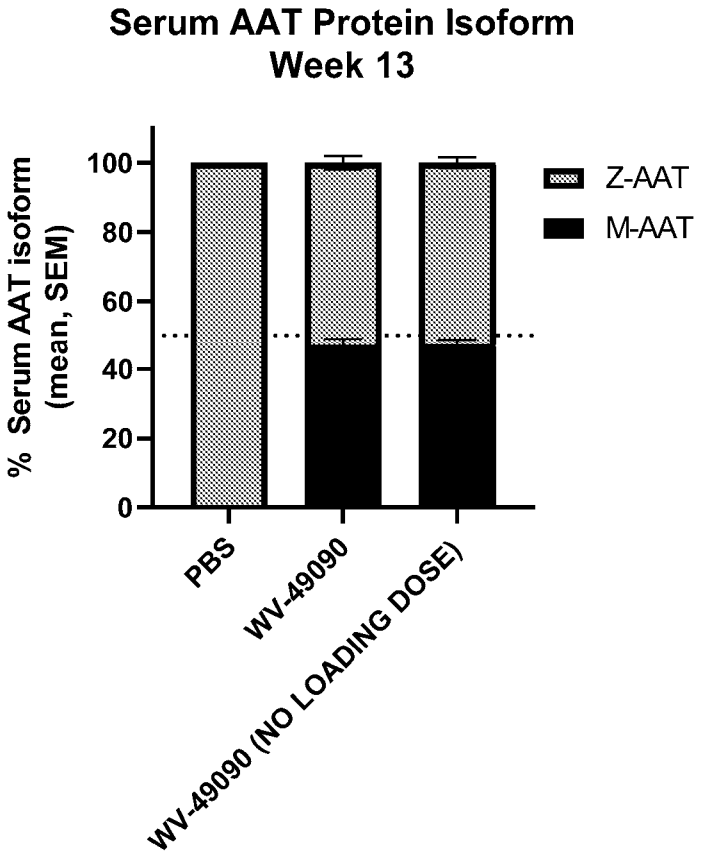

Serum AAT Protein Isoform
Week 13
% Serum AAT isoform
(mean, SEM)
100
80
60
40
20
0
Z-AAT
M-AAT
PBS
WV-49090
WV-49090 (NO LOADING DOSE)

FIG. 14

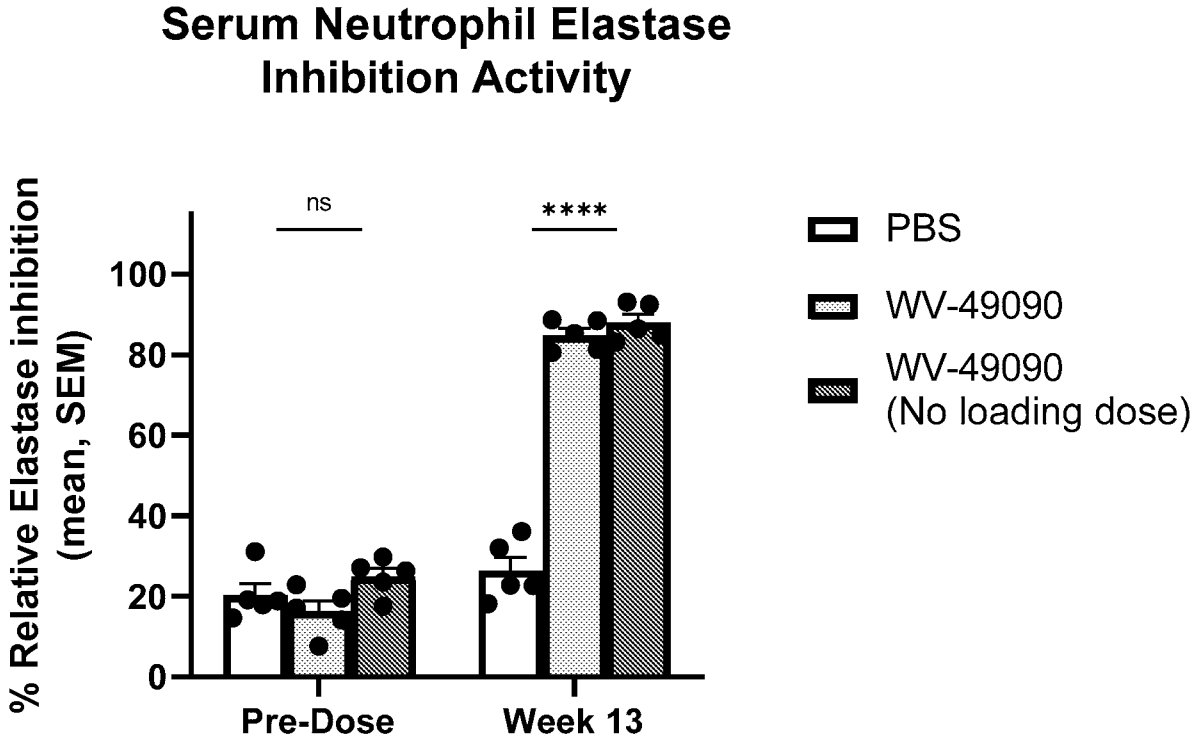

Serum Neutrophil Elastase
Inhibition Activity
% Relative Elastase inhibition
(mean, SEM)
ns
****
100
80
60
40
20
0
Pre-Dose
Week 13
PBS
WV-49090
WV-49090
(No loading dose)

FIG. 15