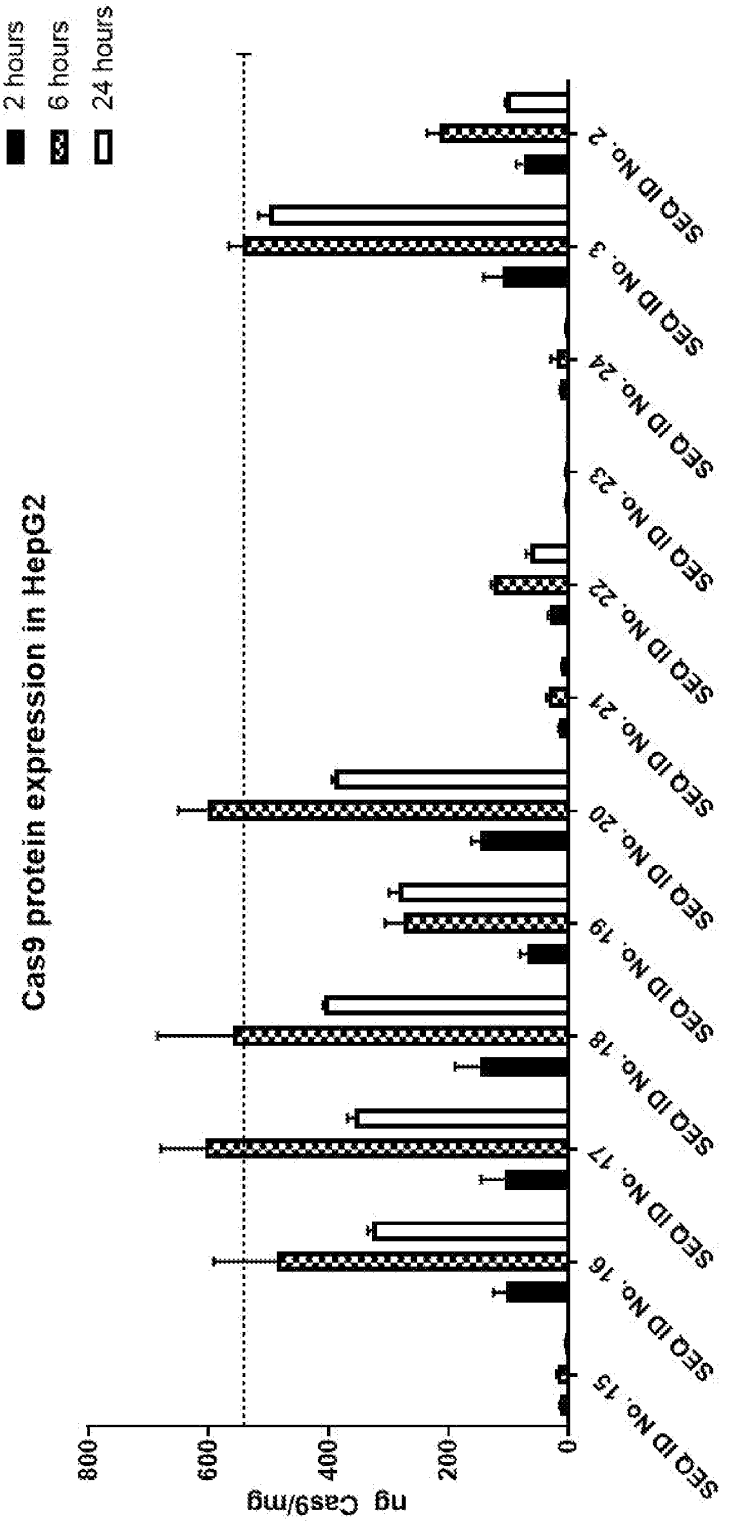

Cas9 protein expression in HepG2
2 hours
6 hours
24 hours
ng Cas9/mg
0
200
400
600
800
SEQ ID No. 15
SEQ ID No. 16
SEQ ID No. 17
SEQ ID No. 18
SEQ ID No. 19
SEQ ID No. 20
SEQ ID No. 21
SEQ ID No. 22
SEQ ID No. 23
SEQ ID No. 24
SEQ ID No. 3
SEQ ID No. 2

Figure 1

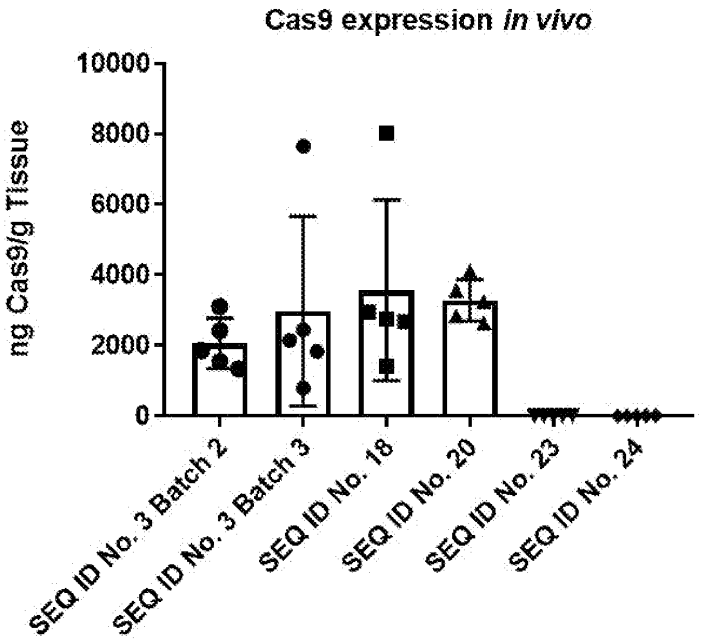


Figure 2

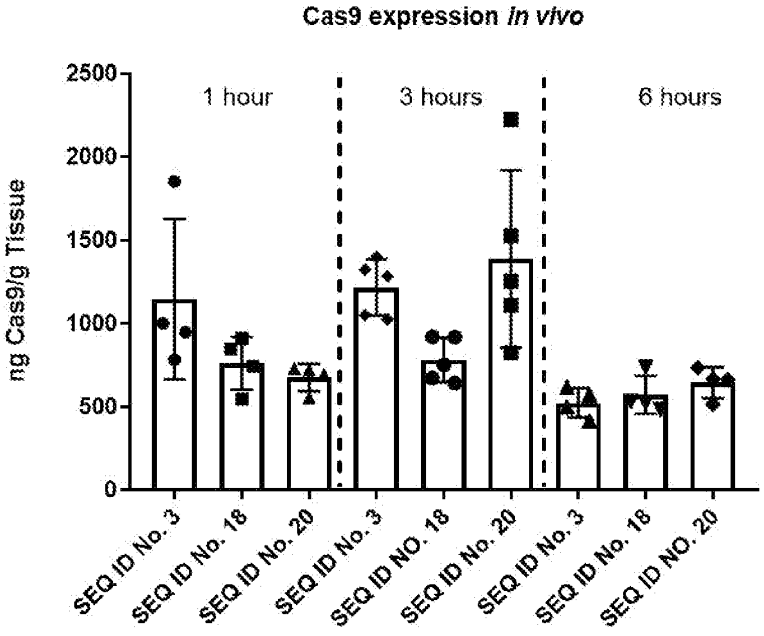


Figure 3

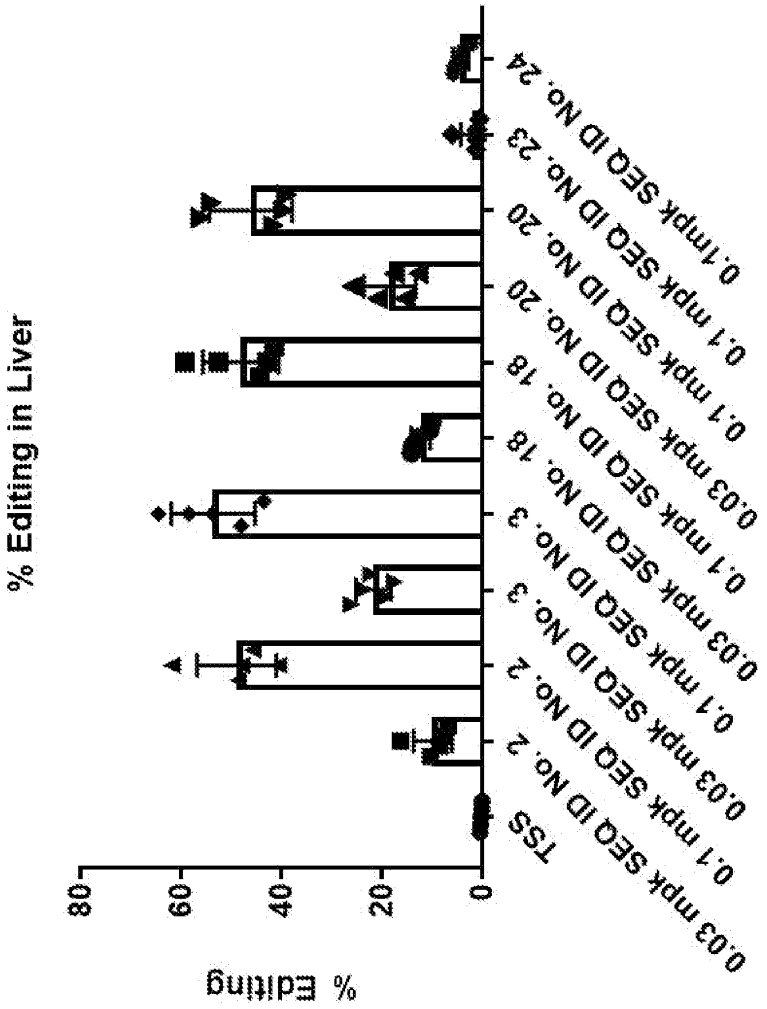

% Editing in Liver
% Editing
0
20
40
60
80
TSS
0.03 mpk SEQ ID No. 2
0.1 mpk SEQ ID No. 2
0.03 mpk SEQ ID No. 3
0.1 mpk SEQ ID No. 3
0.03 mpk SEQ ID No. 18
0.1 mpk SEQ ID No. 18
0.03 mpk SEQ ID No. 20
0.1 mpk SEQ ID No. 20
0.1 mpk SEQ ID No. 23
0.1mpk SEQ ID No. 24

Figure 4A

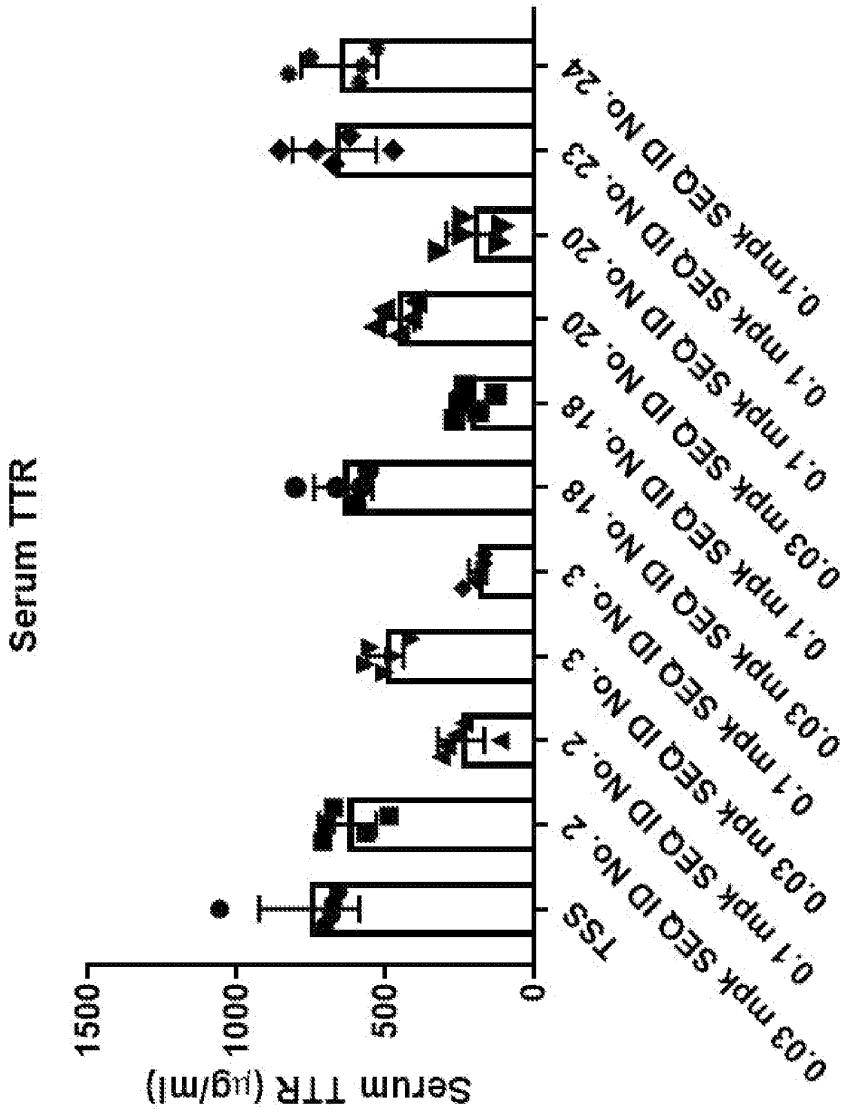

Serum TTR
Serum TTR (µg/ml)
1500
1000
500
0
TSS
0.1 mpk SEQ ID No. 2
0.03 mpk SEQ ID No. 2
0.1 mpk SEQ ID No. 3
0.03 mpk SEQ ID No. 3
0.1 mpk SEQ ID No. 18
0.03 mpk SEQ ID No. 18
0.1 mpk SEQ ID No. 20
0.03 mpk SEQ ID No. 20
0.1 mpk SEQ ID No. 23
0.1mpk SEQ ID No. 24

Figure 4B

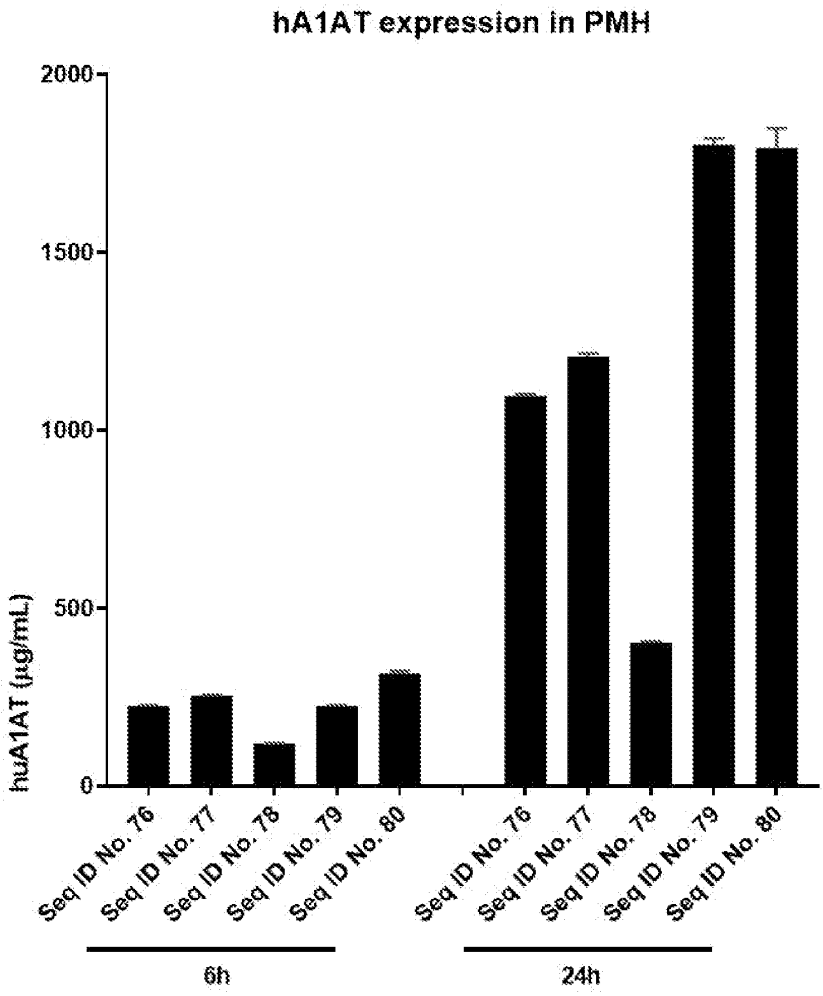

hA1AT expression in PMH
huA1AT (µg/mL)
2000
1500
1000
500
0
Seq ID No. 76
Seq ID No. 77
Seq ID No. 78
Seq ID No. 79
Seq ID No. 80
Seq ID No. 76
Seq ID No. 77
Seq ID No. 78
Seq ID No. 79
Seq ID No. 80
6h
24h

Figure 5A

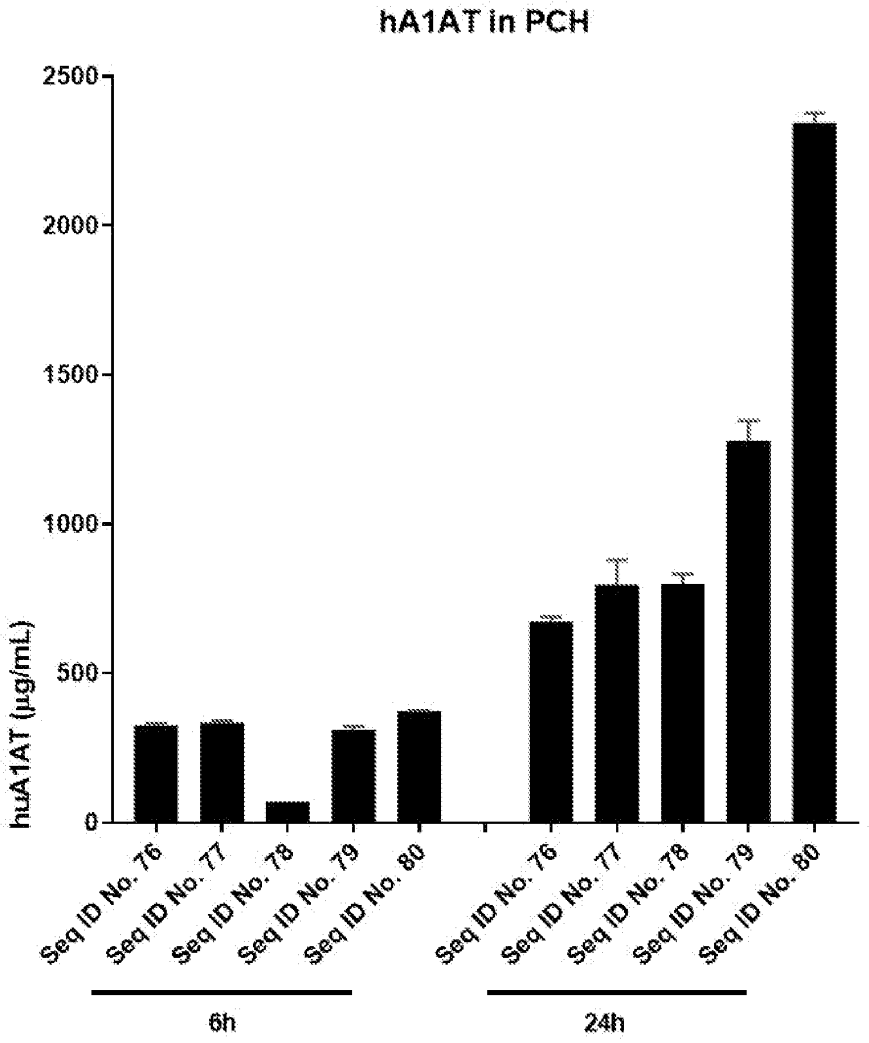

hA1AT in PCH
huA1AT (µg/mL)
2500
2000
1500
1000
500
0
Seq ID No. 76
Seq ID No. 77
Seq ID No. 78
Seq ID No. 79
Seq ID No. 80
Seq ID No. 76
Seq ID No. 77
Seq ID No. 78
Seq ID No. 79
Seq ID No. 80
6h
24h

Figure 5B

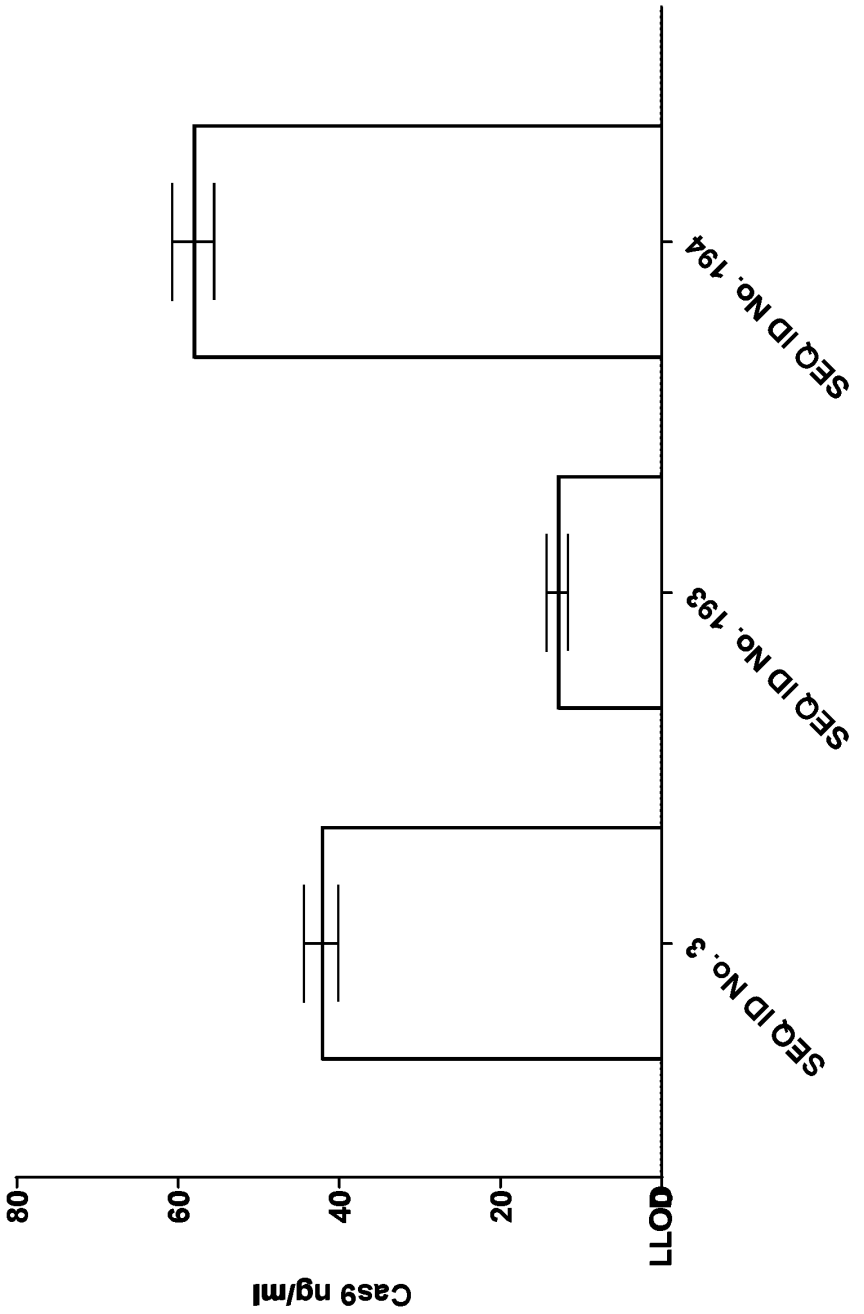


Figure 6

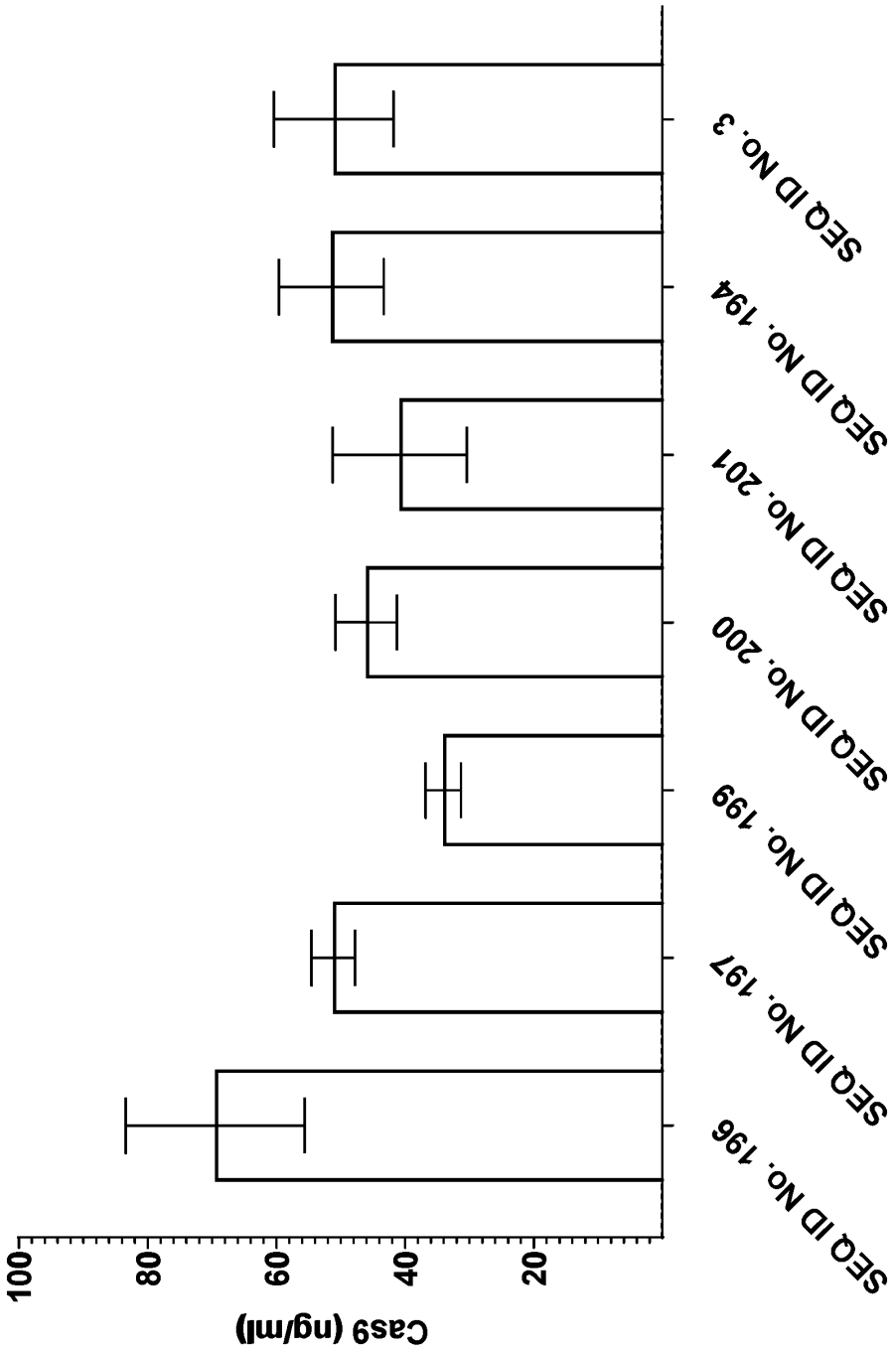

Cas9 (ng/ml)
100
80
60
40
20
SEQ ID No. 196
SEQ ID No. 197
SEQ ID No. 199
SEQ ID No. 200
SEQ ID No. 201
SEQ ID No. 194
SEQ ID No. 3

Figure 7A

Cas9 (ng/ml)
100
80
60
40
20
SEQ ID No. 195
SEQ ID No. 199
SEQ ID No. 197
SEQ ID No. 201
SEQ ID No. 194
SEQ ID No. 3

Figure 7B