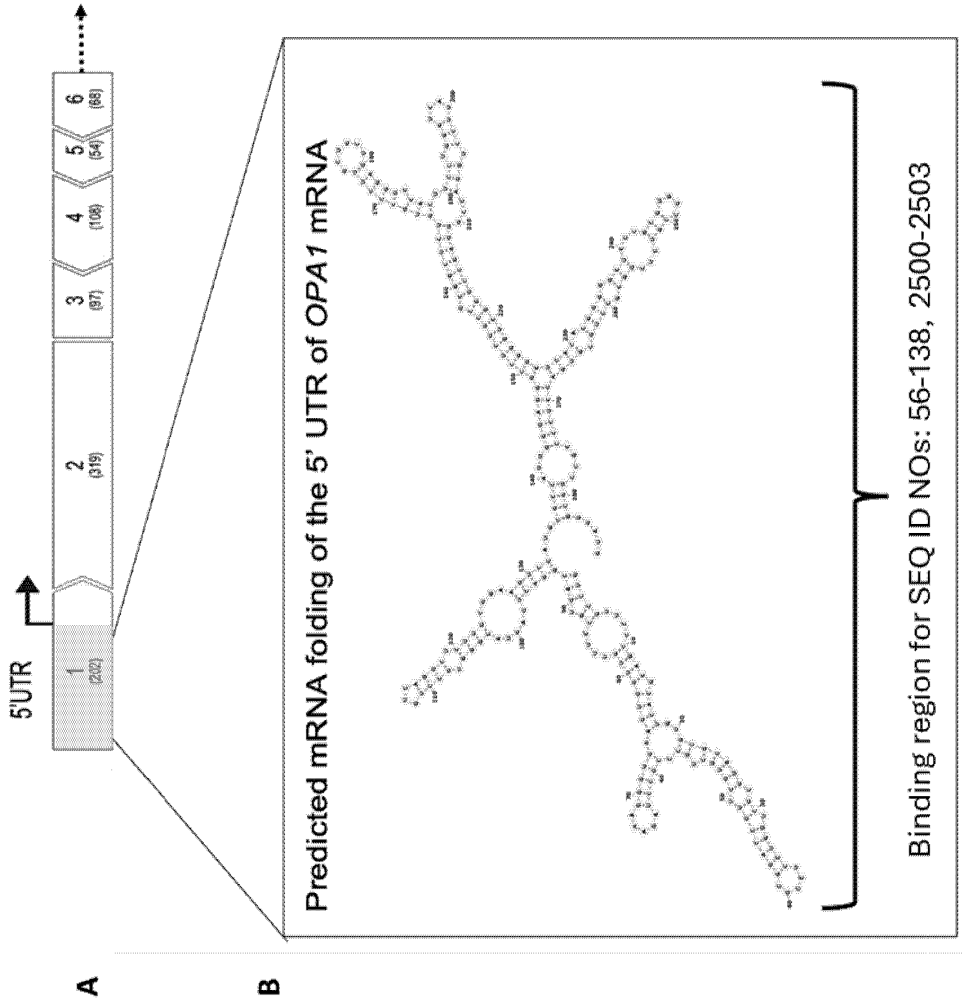

5'UTR
1 (202)
2 (319)
3 (87)
4 (108)
5 (54)
6 (68)
A
B
Predicted mRNA folding of the 5' UTR of OPA1 mRNA
Binding region for SEQ ID NOs: 56-138, 2500-2503

Figure 1

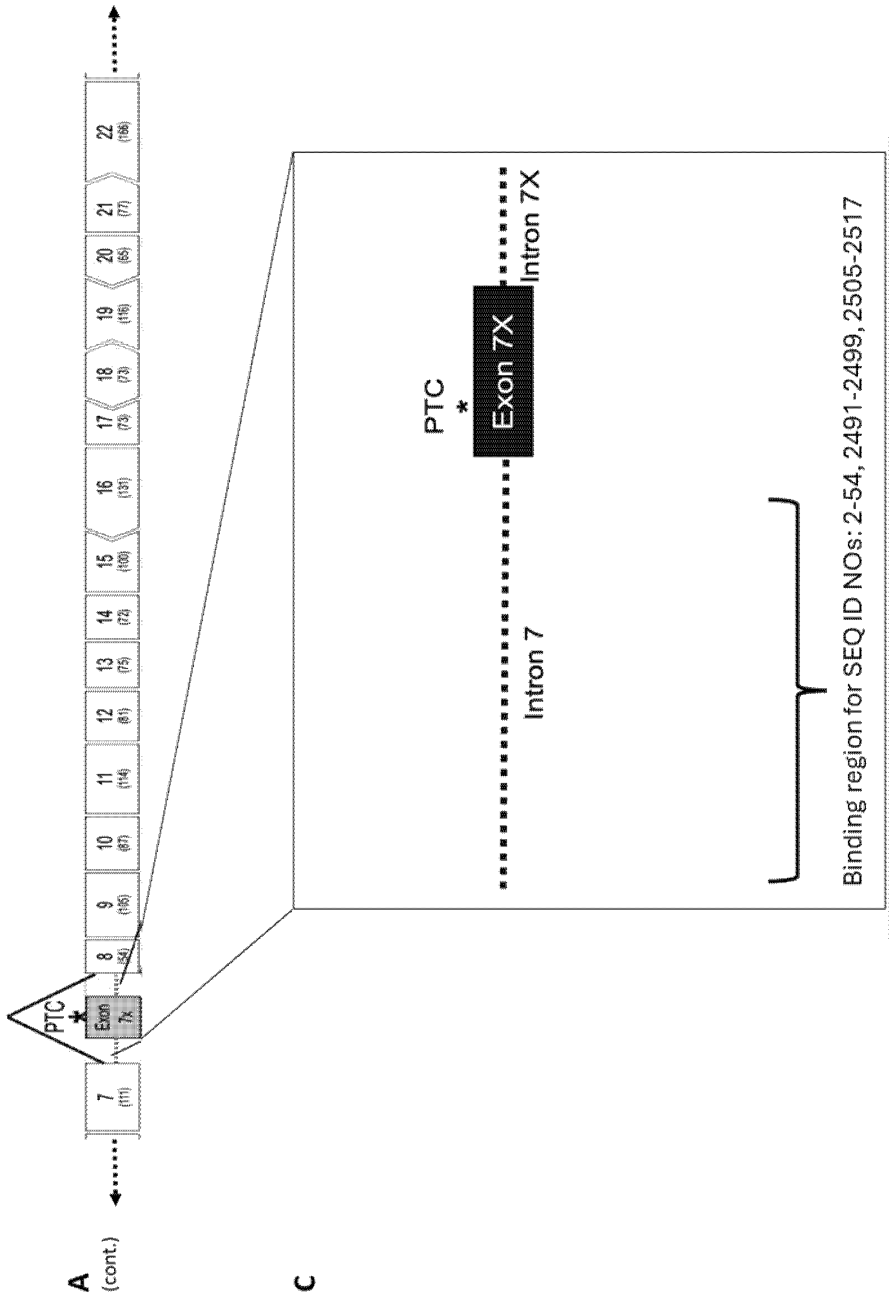


Figure 1 continued

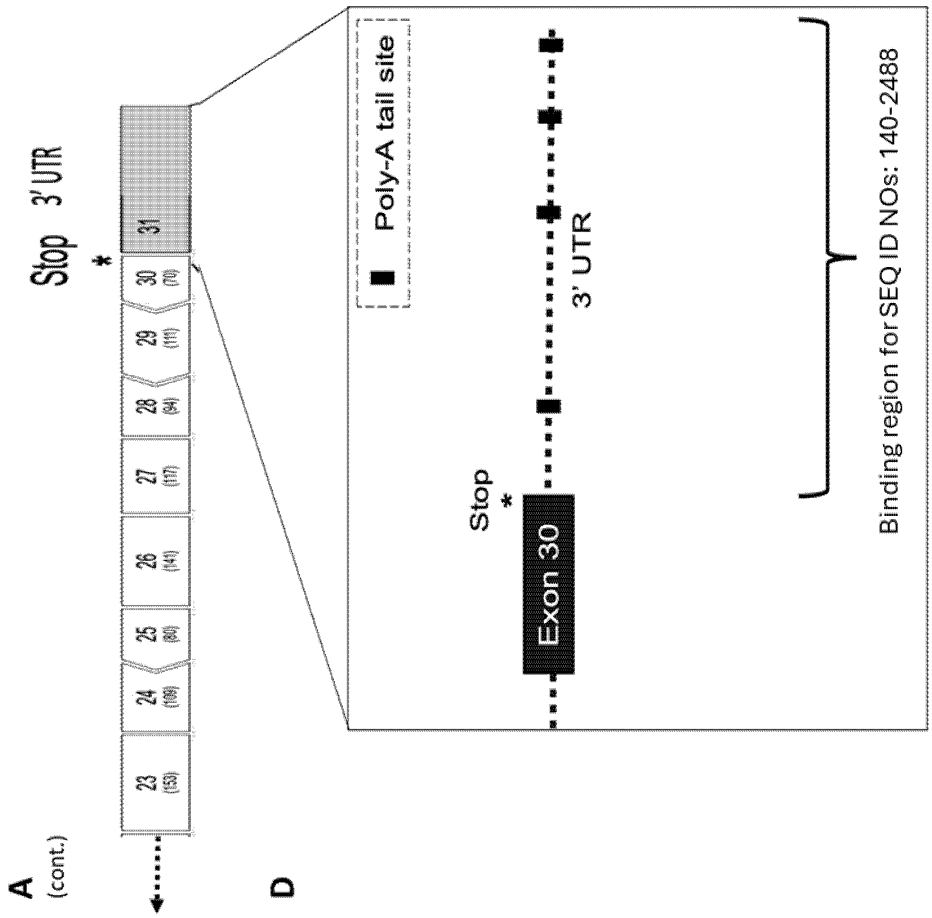


Figure 1 continued

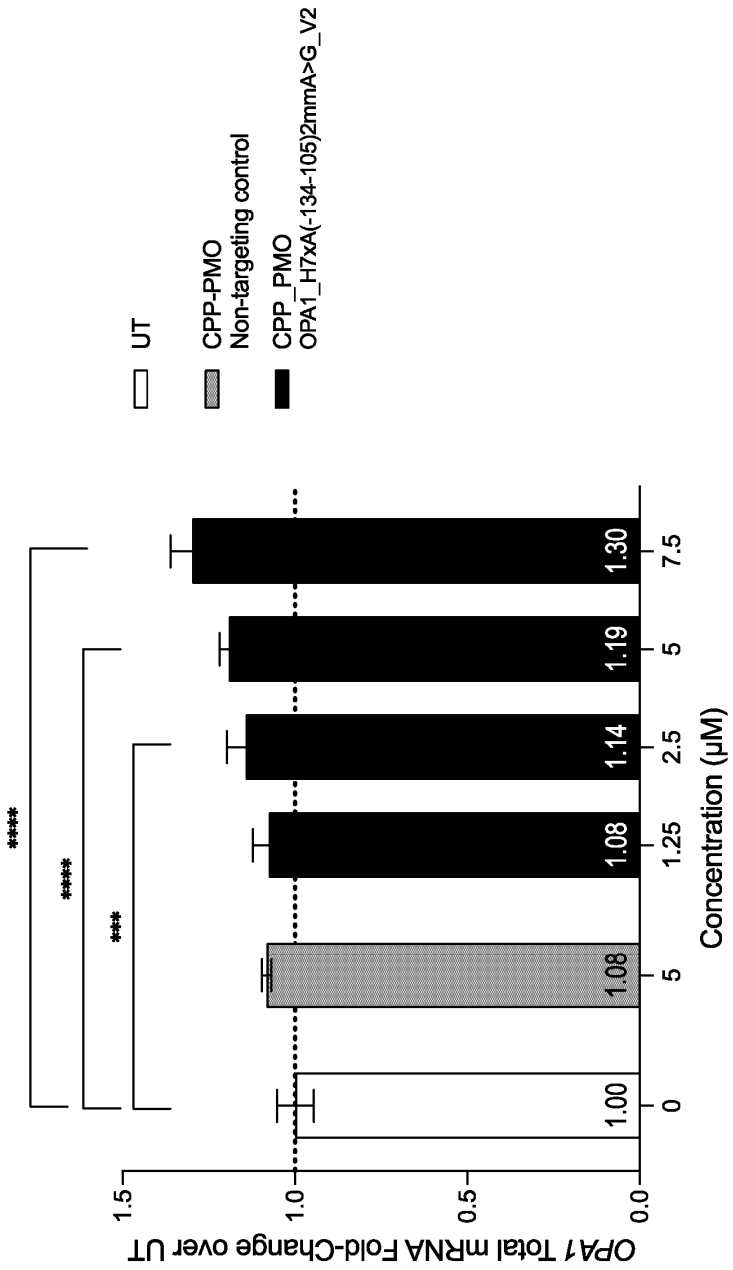

UT
CPP-PMO
Non-targeting control
CPP_PMO
OPA1_H7xA(-134-105)2mmA>G_V2
OPA1 Total mRNA Fold-Change over UT
1.5
1.0
0.5
0.0
1.00
1.08
1.08
1.14
1.19
1.30
0
5
1.25
2.5
5
7.5
Concentration (μM)
***
****
****

Figure 2

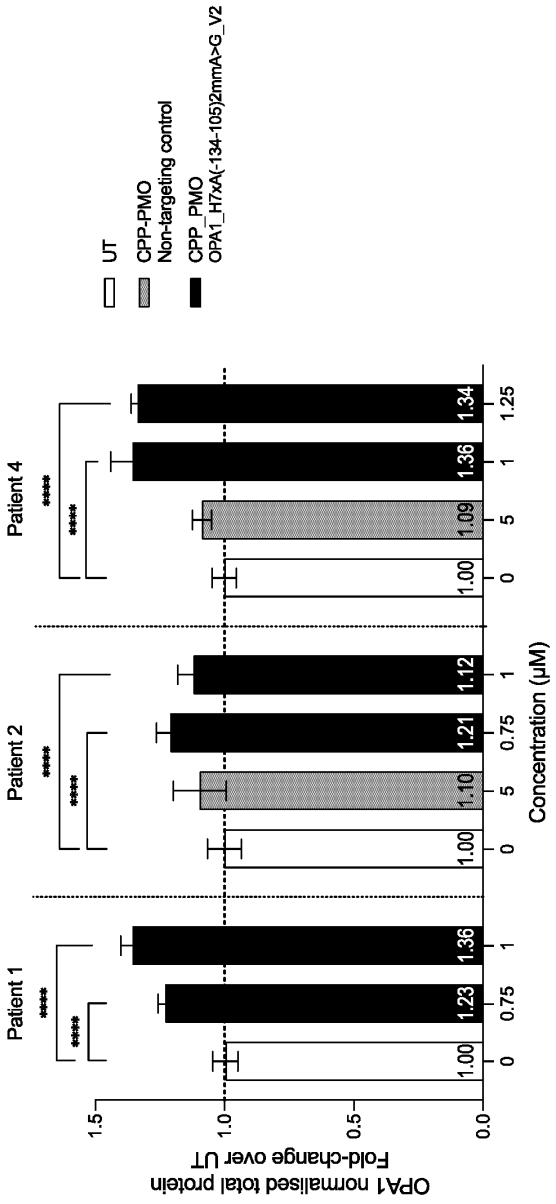

UT
CPP-PMO
Non-targeting control
CPP_PMO
OPA1_H7xA(-134-105)2mmA>G_V2
OPA1 normalised total protein
Fold-change over UT
1.5
1.0
0.5
0.0
Patient 1
Patient 2
Patient 4
****
****
****
****
****
****
1.00
1.23
1.36
1.00
1.10
1.21
1.12
1.00
1.09
1.36
1.34
0
0.75
1
0
5
0.75
1
0
5
1
0
5
1
1.25
Concentration (μM)

Figure 3