

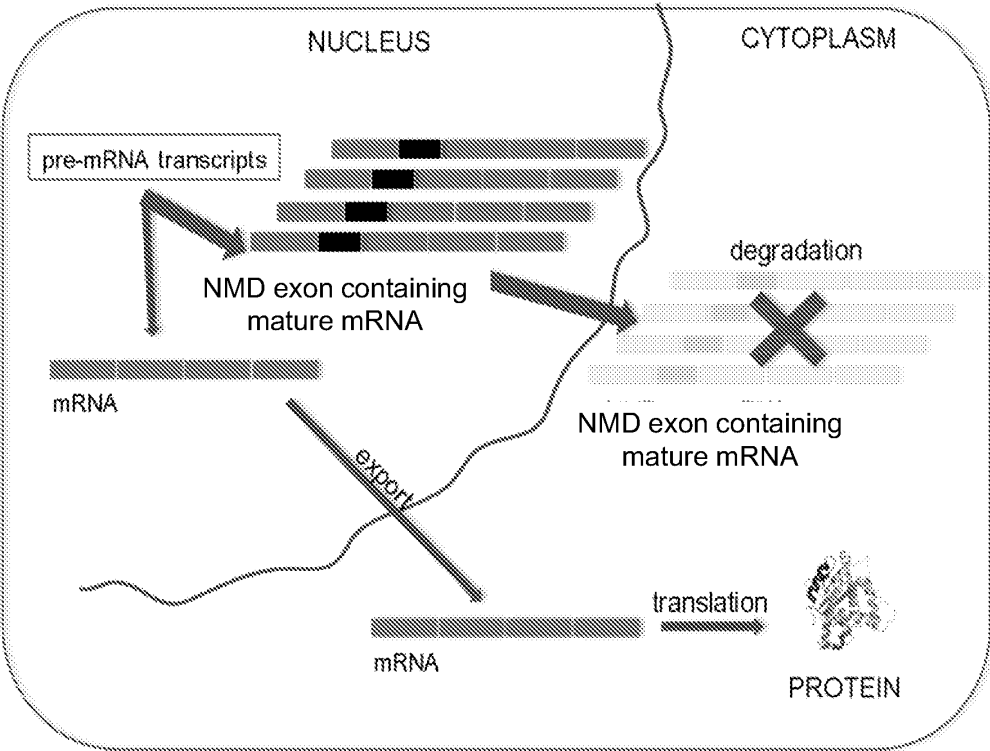


FIG. 1A

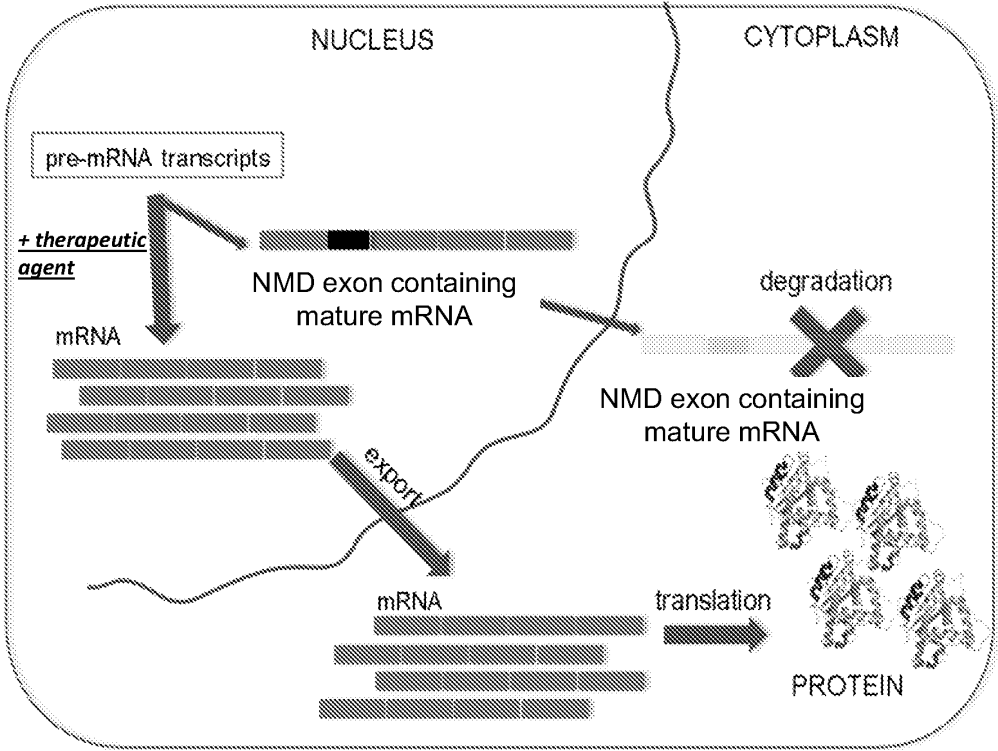


FIG. 1B

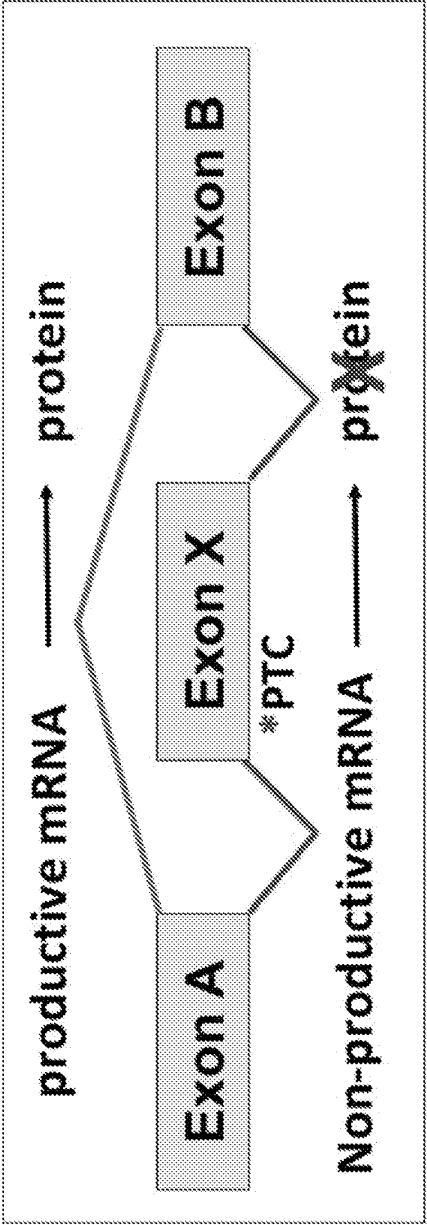


FIG. 1C

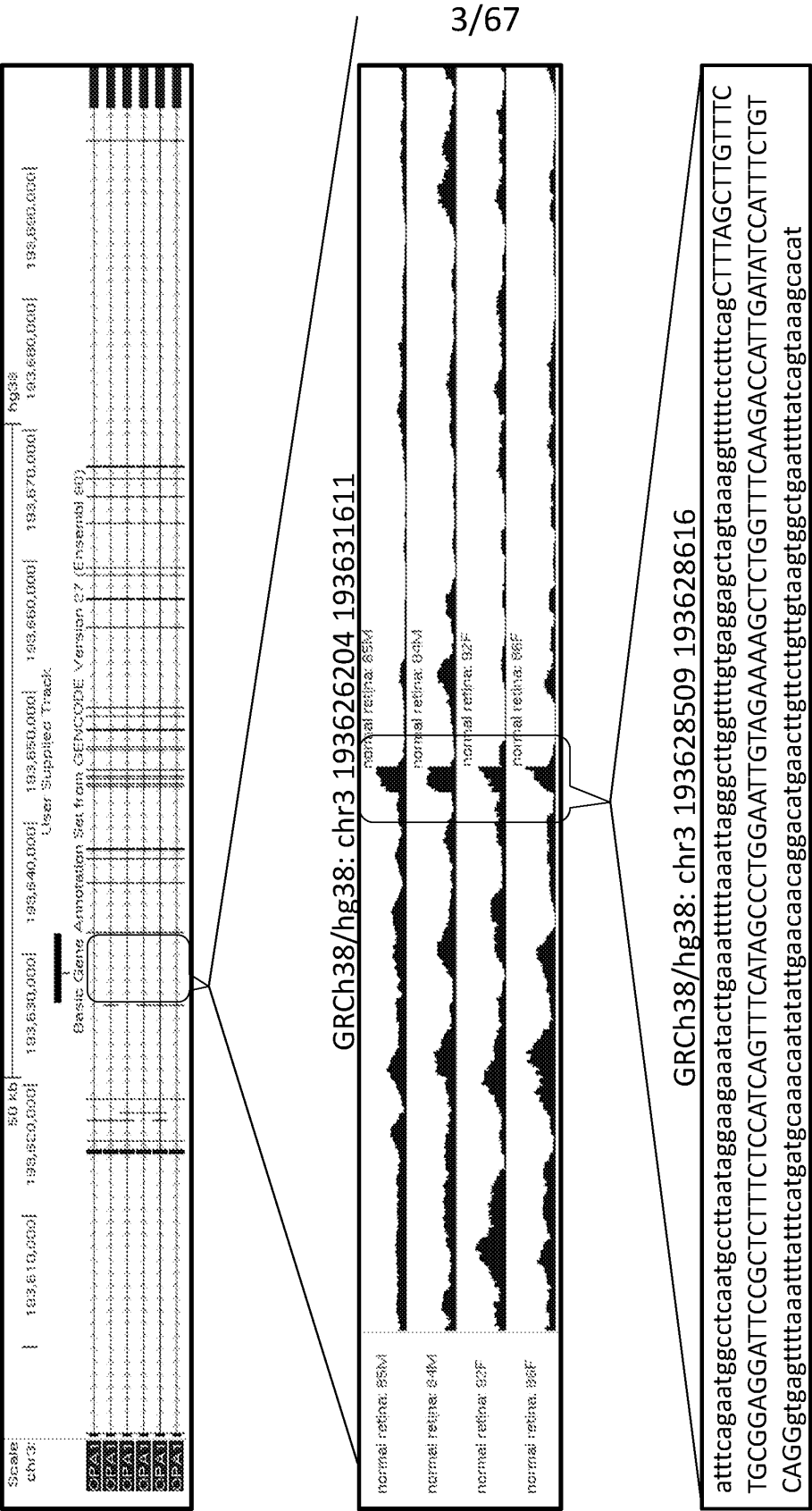


FIG. 2

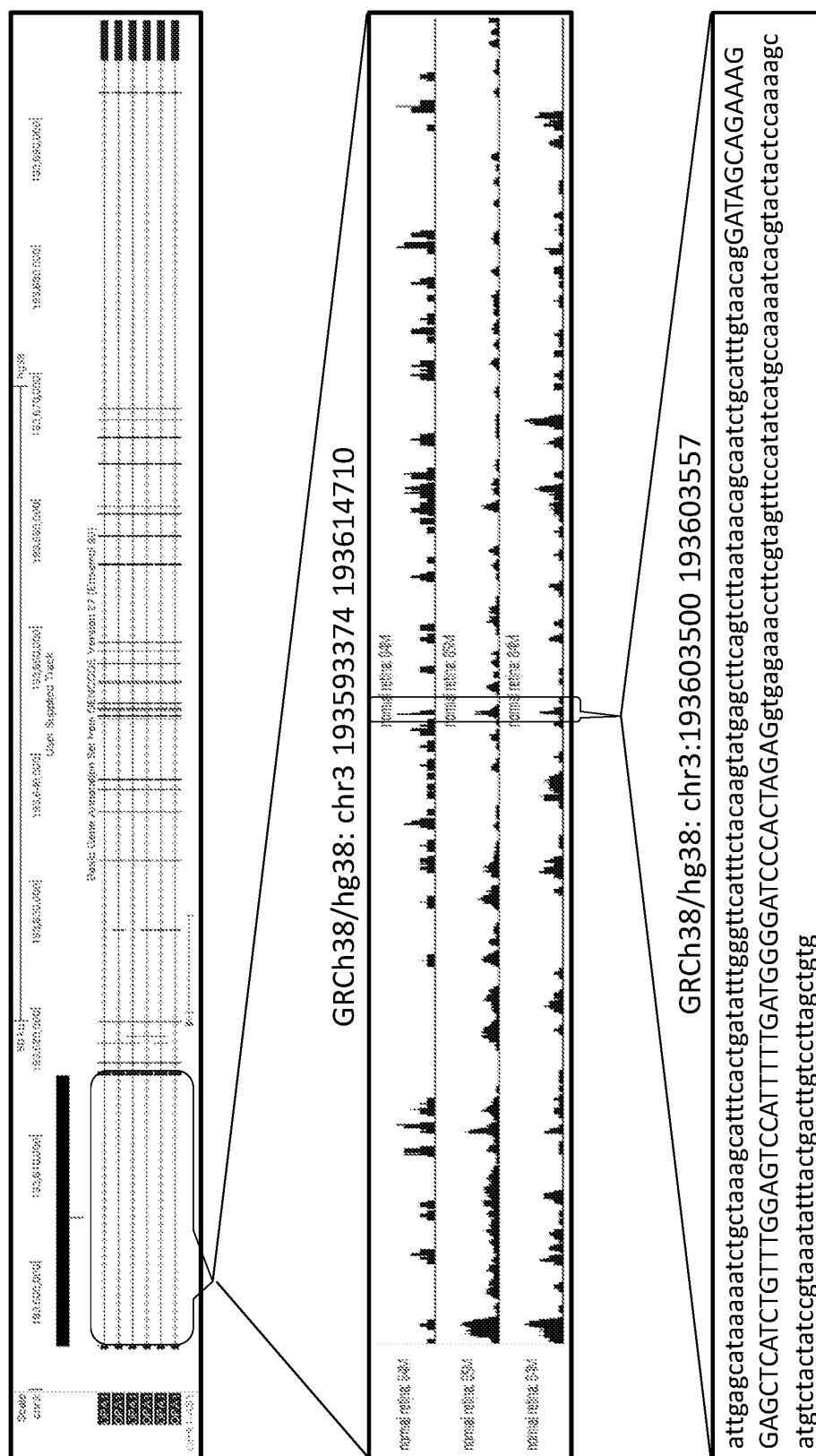


FIG. 3

OPA1
Intron: GRCh38/hg38: chr3 193626204 193631611
Event: GRCh38/hg38: chr3 193628509 193628616 (7X)

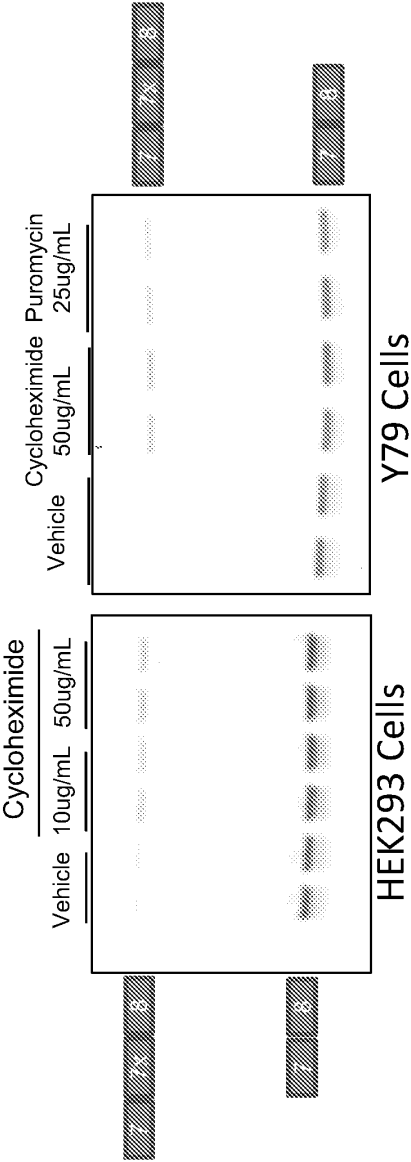
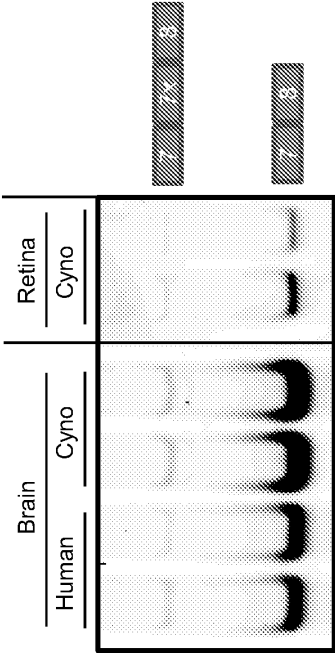


FIG. 4

OPA1
Intron: GRCh38/hg38: chr3 193626204 193631611
Event: GRCh38/hg38: chr3 193628509 193628616 (7X)

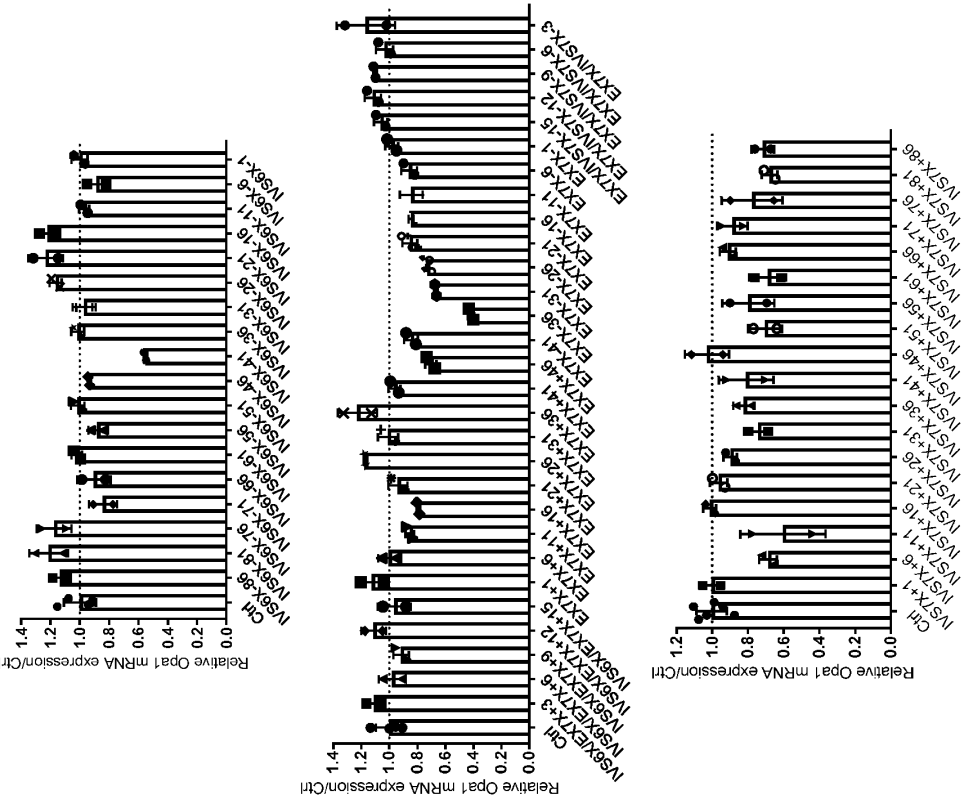


FIG. 6

n=2
Taqman qPCR
HEK293 cells
ASO: 80 nM
Transfection: 24 hrs

OPA1
Intron: GRCh38/hg38: chr3 193626204 193631611
Event: GRCh38/hg38: chr3 193628509 193628616 (7X)

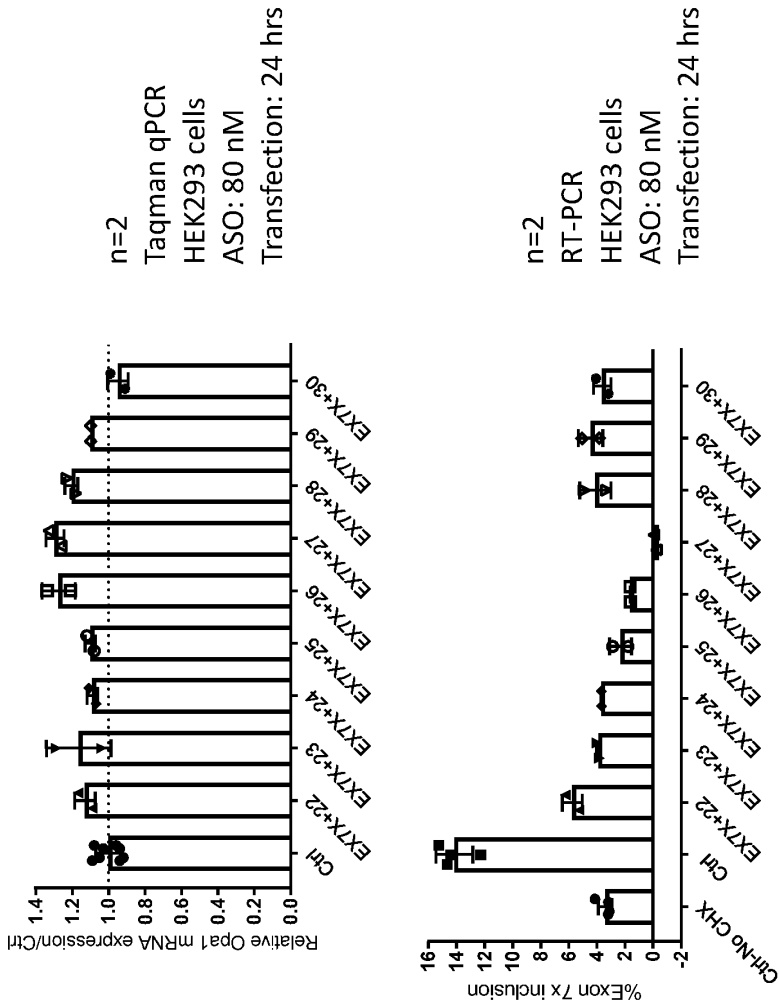


FIG. 7

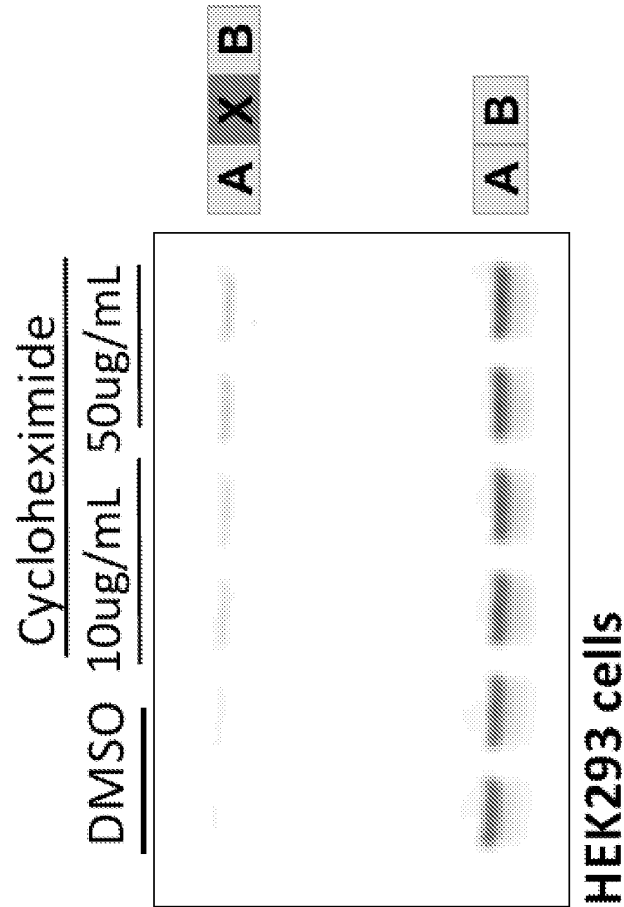


FIG. 8

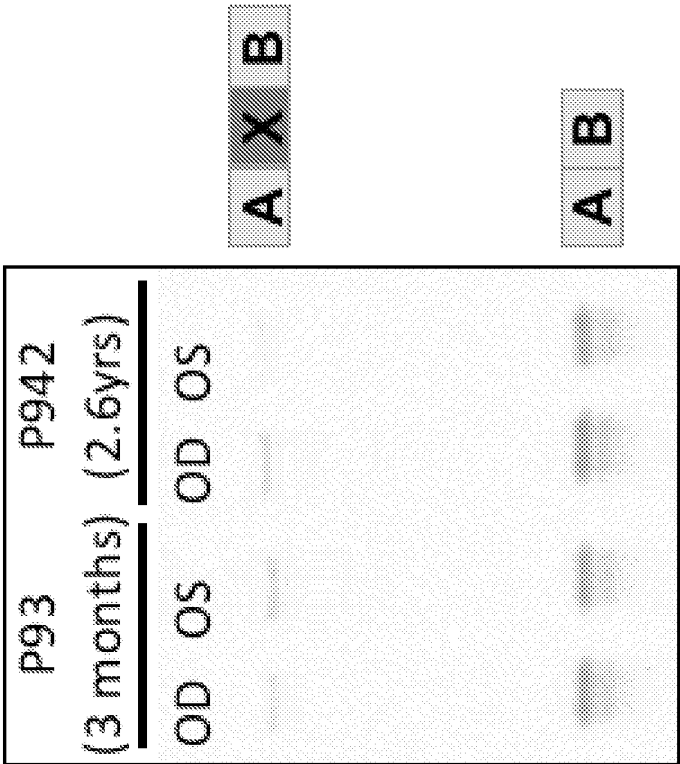


FIG. 9A

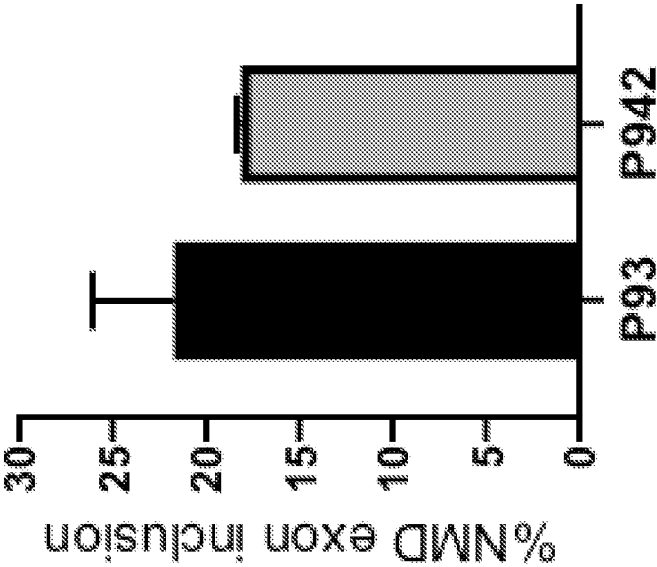


FIG. 9B

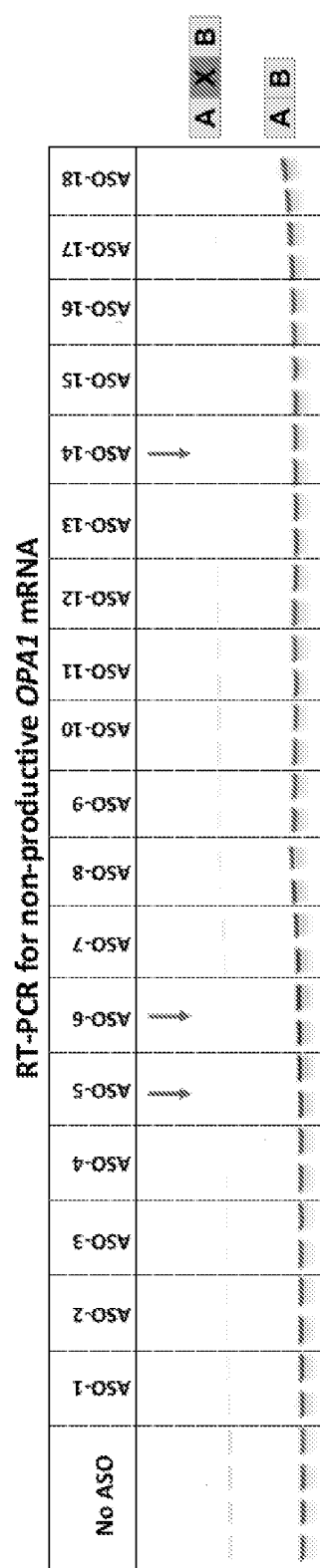


FIG. 10A

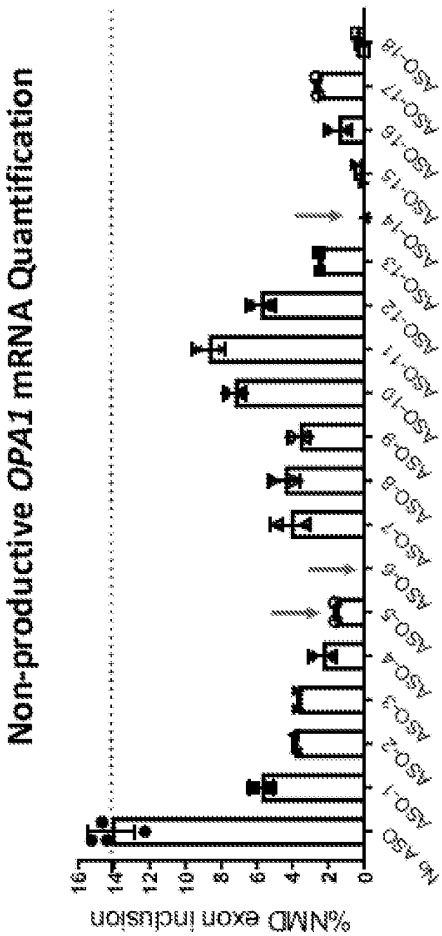


FIG. 10B

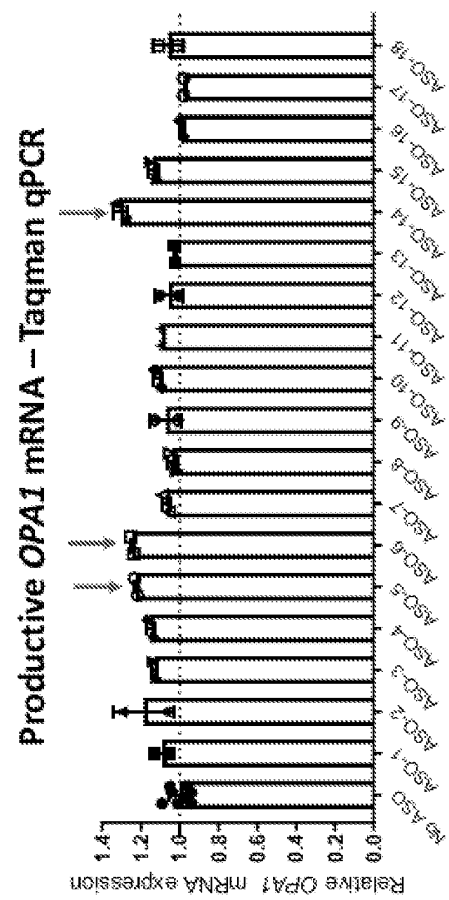


FIG. 11

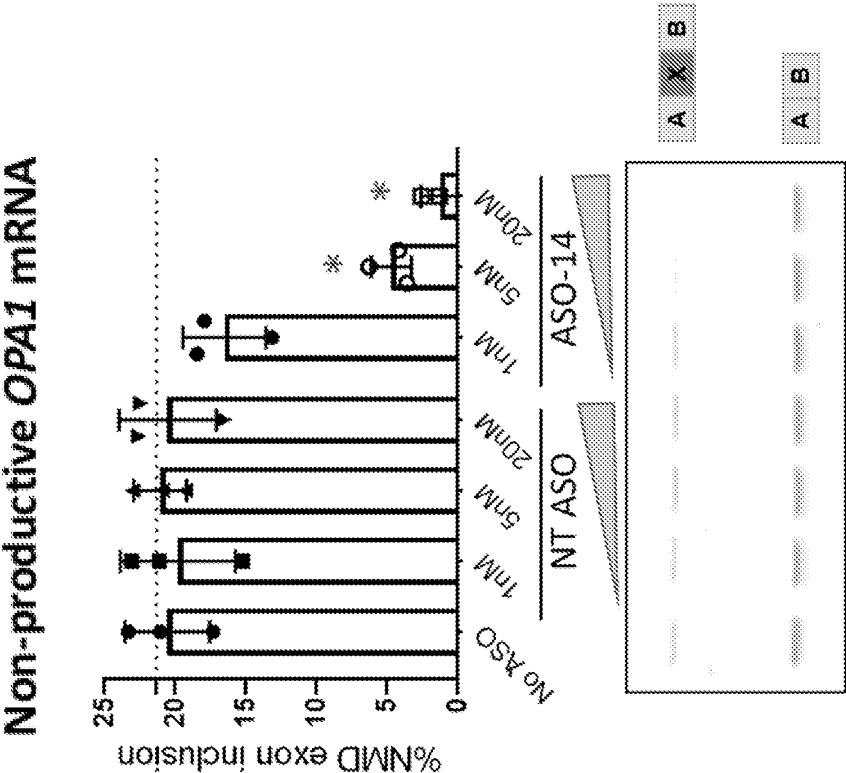


FIG. 12A

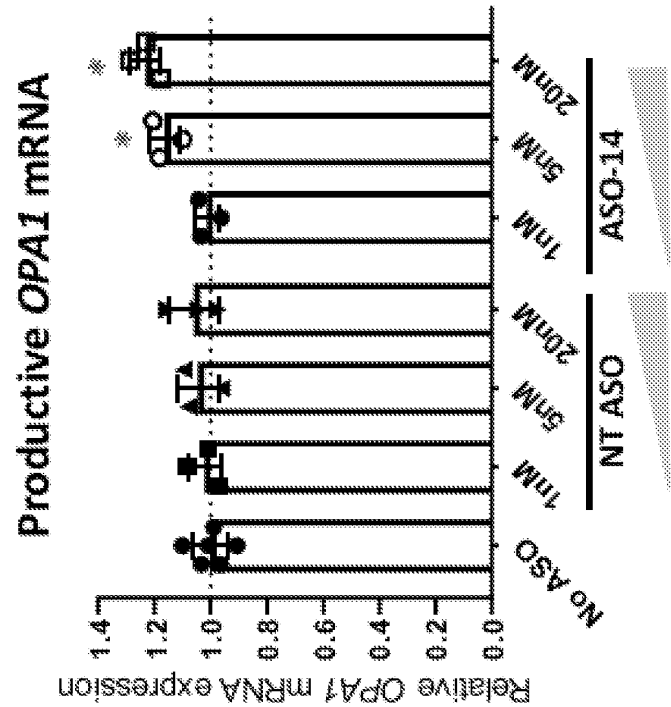


FIG. 12B

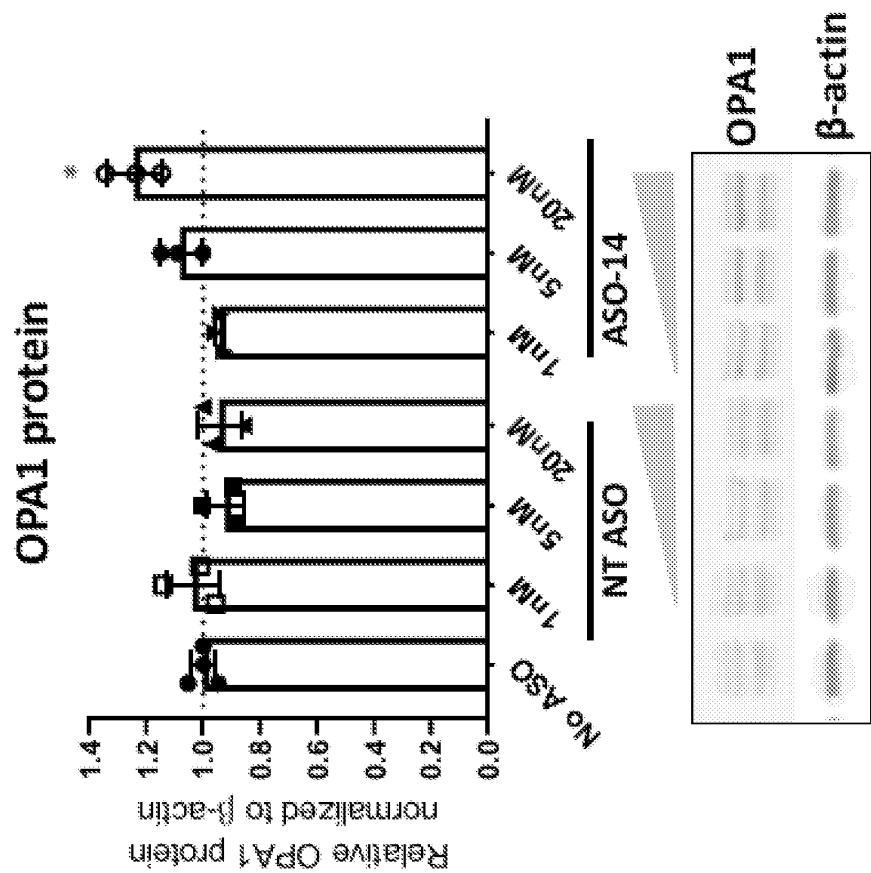


FIG. 12C

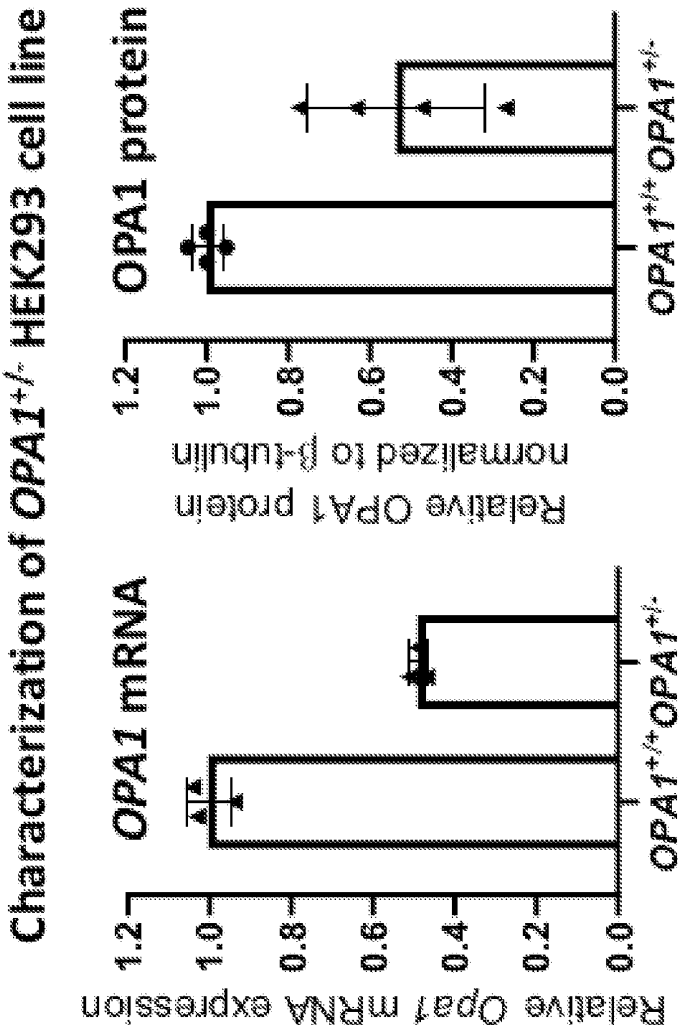


FIG. 13A

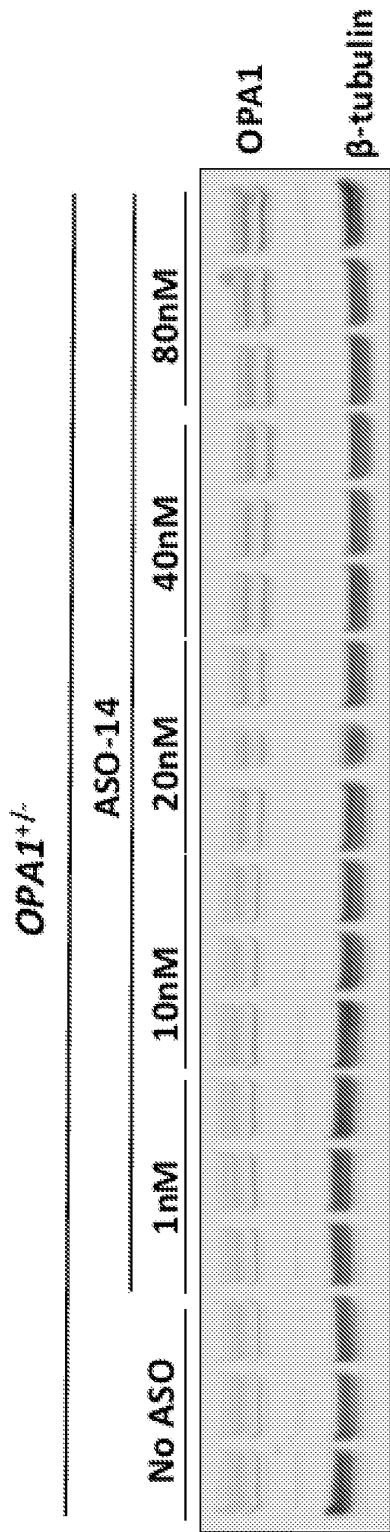


FIG. 13B

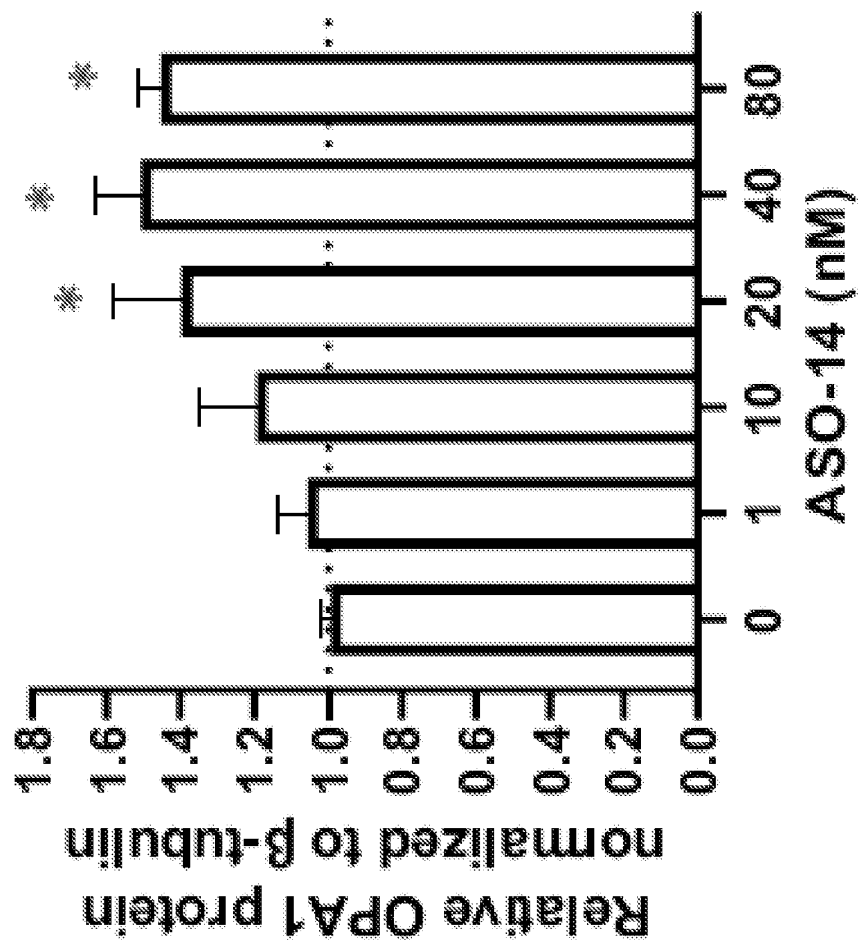


FIG. 13C

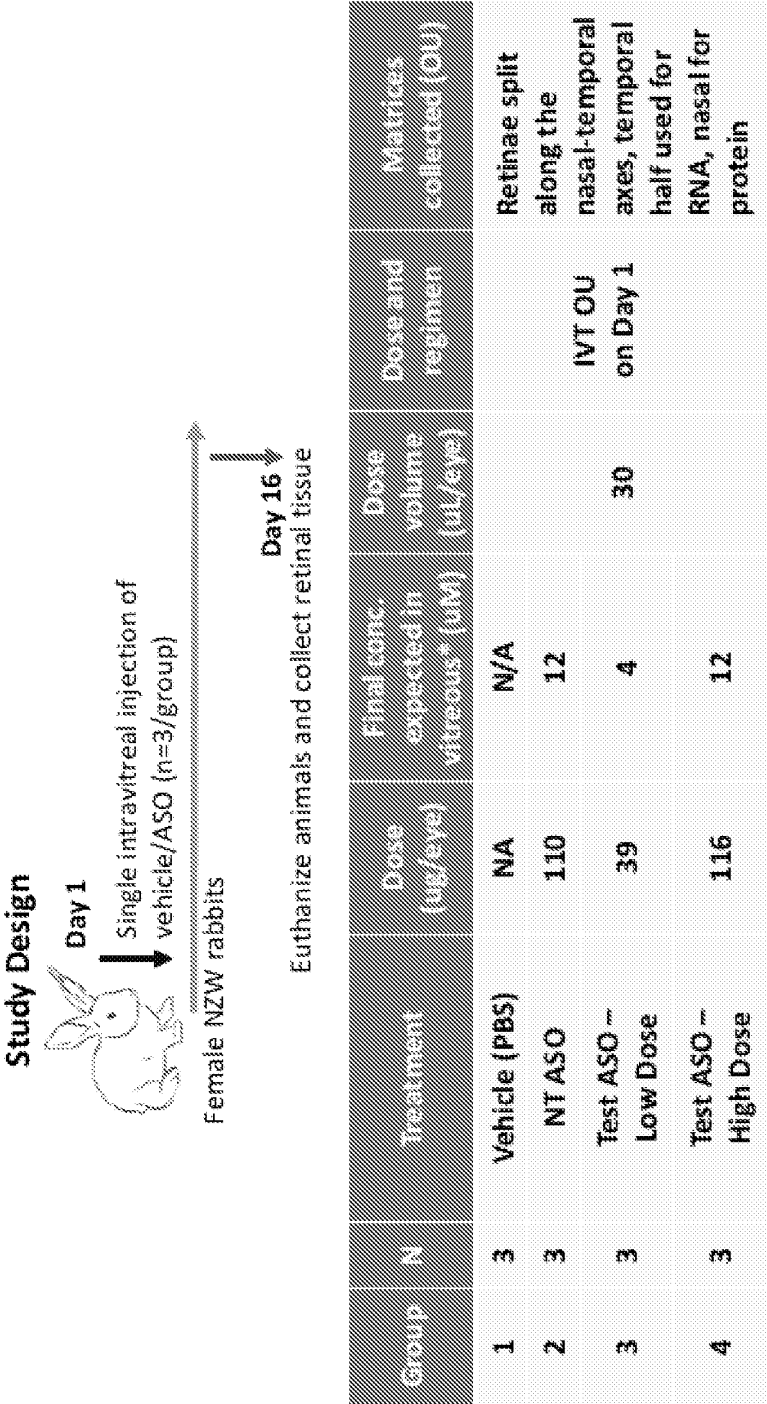


FIG. 14A

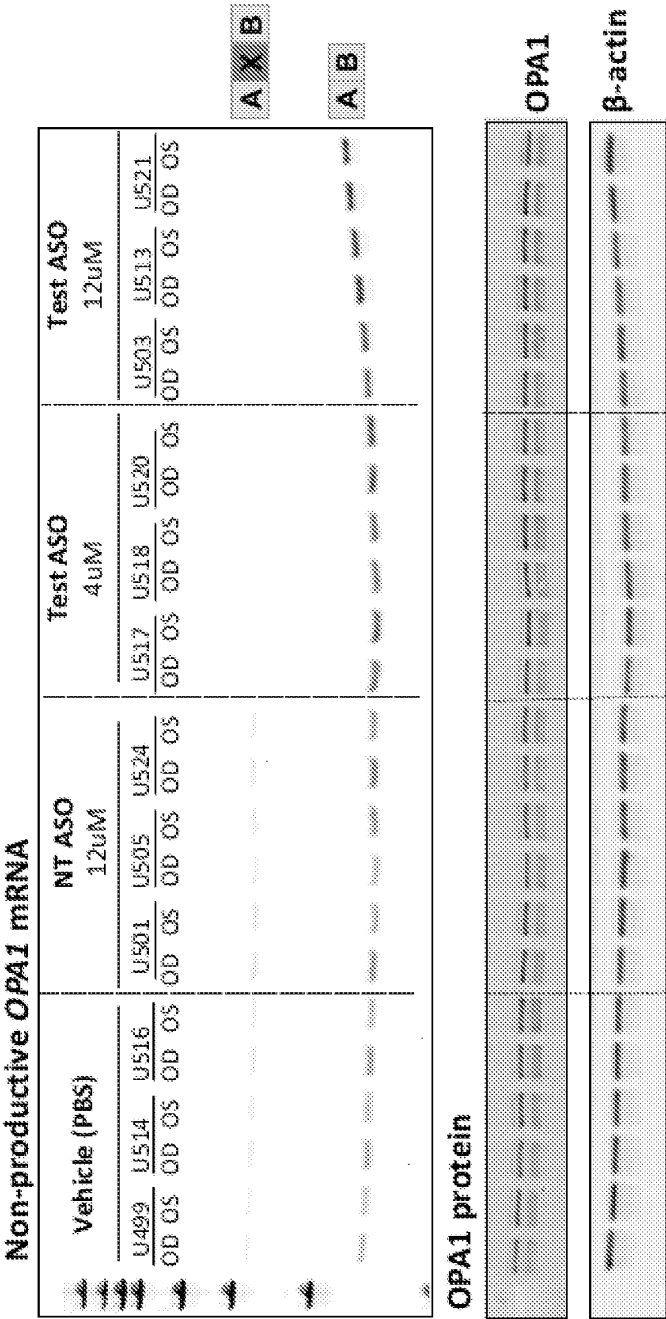


FIG. 14B

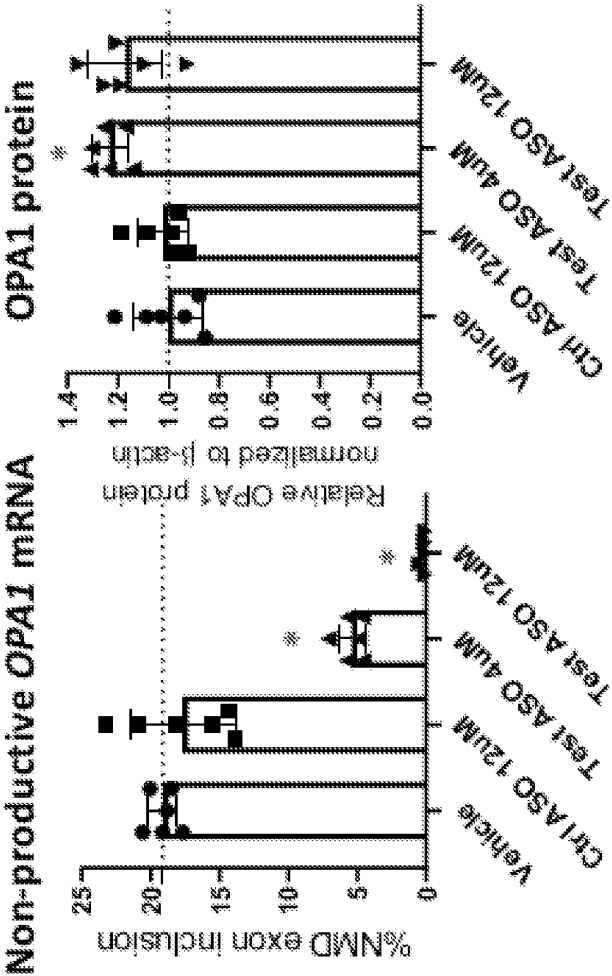


FIG. 14C

Compound ID No.:		Sequence (5'-3')	Chemistry
			(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
1	AGCCATTCTGAAATCT	AGGCCATCTGAAATCT	
2	CATTGAGGCCATTCTGAA	CATTGAGGCCATTCTGAA	
3	TAAGGCATTGAGGCCATT	TAAGGCATTGAGGCCATT	
4	CCTATTAAAGGCATTGAGG	CCTATTAAAGGCATTGAGG	
5	TTCTTCCTATTAAAGGCAT	TTCTTCCTATTAAAGGCAT	
6	AGTATTCTTCCTATTAA	AGTATTCTTCCTATTAA	
7	TTTCAAGTATTCTTCCT	TTTCAAGTATTCTTCCT	
8	AAAAATTCAAGTATTTC	AAAAATTCAAGTATTTC	
9	AATTTAAAAATTCAAGT	AATTTAAAAATTCAAGT	
10	GCCCTAATTAAAAATTT	GCCCTAATTAAAAATTT	
11	ACCAAGCCCTAATTAAAA	ACCAAGCCCTAATTAAAA	
12	ACAAAACCAAGCCCTAAT	ACAAAACCAAGCCCTAAT	
13	TCCTCACAAAAACCAAGCC	TCCTCACAAAAACCAAGCC	
14	CTAGCTCCTCACAAAACC	CTAGCTCCTCACAAAACC	
15	CTTTACTAGCTCCTCACA	CTTTACTAGCTCCTCACA	
16	AAAACCTTTACTAGCTCC	AAAACCTTTACTAGCTCC	
17	AGAGAAAAACCTTTACTA	AGAGAAAAACCTTTACTA	
18	CTGAAAAGAGAAAAACCTT	CTGAAAAGAGAAAAACCTT	
19	AAGCTGAAAAGAGAAAAAC	AAGCTGAAAAGAGAAAAAC	
20	CTAAAGCTGAAAAGAGAAA	CTAAAGCTGAAAAGAGAAA	

FIG. 15

Compound ID No.:	Sequence (5'-3')	Chemistry	
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
21	AAGCTAAAGCTGAAAGAG	AAGCTAAAGCTGAAAGAG	
22	AACAAGCTAAAGCTGAAA	AACAAGCTAAAGCTGAAA	
23	AGAAACAAGCTAAAGCTG	AGAAACAAGCTAAAGCTG	
24	CGCAGAAACAAGCTAAAG	CGCAGAAACAAGCTAAAG	
25	TCCTCCGAGAAACAAGC	TCCTCCGAGAAACAAGC	
26	CGGAATCCTCCGAGAAA	CGGAATCCTCCGAGAAA	
27	AAGAGCGGAATCCTCCGC	AAGAGCGGAATCCTCCGC	
28	GGAGAAAGAGCGGAATCC	GGAGAAAGAGCGGAATCC	
29	CTGATGGAGAAAGAGCGG	CTGATGGAGAAAGAGCGG	
30	TGAAACTGATGGAGAAAG	TGAAACTGATGGAGAAAG	
31	GGCTATGAAACTGATGGA	GGCTATGAAACTGATGGA	
32	TCCAGGGCTATGAAACTG	TCCAGGGCTATGAAACTG	
33	ACAATCCAGGGCTATGA	ACAATCCAGGGCTATGA	
34	TTTCTACAATCCAGGGC	TTTCTACAATCCAGGGC	
35	GAGCTTTTCTACAATCC	GAGCTTTTCTACAATCC	
36	AACCAGAGCTTTTCTACA	AACCAGAGCTTTTCTACA	
37	CTTGAAACCAGAGCTTTT	CTTGAAACCAGAGCTTTT	
38	ATGGTCTTGAAACCAGAG	ATGGTCTTGAAACCAGAG	
39	TATCAATGGTCTTGAAAC	TATCAATGGTCTTGAAAC	
40	ATGGATATCAATGGTCTT	ATGGATATCAATGGTCTT	

FIG. 15 cont.

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Compound ID No.:	Sequence (5'-3')	Chemistry	
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
41	CAGAAATGGATATCAATG	CAGAAATGGATATCAATG	
42	CCTGACAGAAATGGATAT	CCTGACAGAAATGGATAT	
43	CACCCTGACAGAAATGGA	CACCCTGACAGAAATGGA	
44	ACTCACCCCTGACAGAAAT	ACTCACCCCTGACAGAAAT	
45	AAAACCTCACCCCTGACAGA	AAAACCTCACCCCTGACAGA	
46	TTTAAAACTCACCCCTGAC	TTTAAAACTCACCCCTGAC	
47	AAATTTAAAACTCACCCCT	AAATTTAAAACTCACCCCT	
48	AATAAAATTTAAAACTCAC	AATAAAATTTAAAACTCAC	
49	CATGAAATAAATTTAAAA	CATGAAATAAATTTAAAA	
50	TGCATCATGAAATAAATTT	TGCATCATGAAATAAATTT	
51	TTGTTTGCATCATGAAATTT	TTGTTTGCATCATGAAATTT	
52	ATATATTTGTTGCATCAT	ATATATTTGTTGCATCAT	
53	GTTCAATATATTTGTTGC	GTTCAATATATTTGTTGC	
54	CTGTTGTTCAATATATTTG	CTGTTGTTCAATATATTTG	
55	ATGTCCTGTTGTTCAATA	ATGTCCTGTTGTTCAATA	
56	AGTTCATGTCCTGTTGTT	AGTTCATGTCCTGTTGTT	
57	GAACAAGTTCATGTCCTG	GAACAAGTTCATGTCCTG	
58	AACAAGAACAAGTTCATG	AACAAGAACAAGTTCATG	
59	CTTACAACAAGAACAAGT	CTTACAACAAGAACAAGT	
60	AGCCACTTACAACAAGAA	AGCCACTTACAACAAGAA	

FIG. 15 cont.

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		Chemistry	
Compound ID No.:	Sequence (5'-3')	(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
61	AATTCAGCCACTTACAAC	AAATCAGCCACTTACAAC	
62	GATAAAATTCAGCCACTT	GATAAAATTCAGCCACTT	
63	TTACTGATAAAAATTCAGC	TTACTGATAAAAATTCAGC	
64	GTGCTTTACTGATAAAAT	GTGCTTTACTGATAAAAT	
65	TTGATGTGCTTTACTGAT	TTGATGTGCTTTACTGAT	
66	TGGAGAAAGAGCGGAATC	TGGAGAAAGAGCGGAATC	
67	ATGGAGAAAGAGCGGAAT	ATGGAGAAAGAGCGGAAT	
68	GATGGAGAAAGAGCGGAA	GATGGAGAAAGAGCGGAA	
69	TGATGGAGAAAGAGCGGA	TGATGGAGAAAGAGCGGA	
70	ACTGATGGAGAAAGAGCGG	ACTGATGGAGAAAGAGCGG	
71	AACTGATGGAGAAAGAGC	AACTGATGGAGAAAGAGC	
72	AACTGATGGAGAAAGAG	AACTGATGGAGAAAGAG	
73	GAAACTGATGGAGAAAGA	GAAACTGATGGAGAAAGA	
74	ATGAAACTGATGGAGAAA	ATGAAACTGATGGAGAAA	
75	TATGAAACTGATGGAGAA	TATGAAACTGATGGAGAA	
76	CTATGAAACTGATGGAGA	CTATGAAACTGATGGAGA	
77	GCTATGAAACTGATGGAG	GCTATGAAACTGATGGAG	
78	GGGCTATGAAACTGATGG	GGGCTATGAAACTGATGG	
79	AGGGCTATGAAACTGATG	AGGGCTATGAAACTGATG	
80	CAGGGCTATGAAACTGAT	CAGGGCTATGAAACTGAT	

FIG. 15 cont.

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Compound ID No.:		Sequence (5'-3')	Chemistry
			(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
81	CCAGGGCTATGAAACTGA	CCAGGGCTATGAAACTGA	
82	CTGATGGAGAAAGAGCGGAATC	CTGATGGAGAAAGAGCGGAATC	
83	CTGATGGAGAAAGAGCGGAA	CTGATGGAGAAAGAGCGGAA	
84	AACTGATGGAGAAAGAGCGGAA	AACTGATGGAGAAAGAGCGGAA	
85	AACTGATGGAGAAAGAGCGGG	AACTGATGGAGAAAGAGCGGG	
86	GAAACTGATGGAGAAAGAGCGGG	GAAACTGATGGAGAAAGAGCGGG	
87	GGCTATGAAACTGATGGAGAAA	GGCTATGAAACTGATGGAGAAA	
88	GGCTATGAAACTGATGGAGA	GGCTATGAAACTGATGGAGA	
89	AGGGCTATGAAACTGATGGAGA	AGGGCTATGAAACTGATGGAGA	
90	AGGGCTATGAAACTGATGGGA	AGGGCTATGAAACTGATGGGA	
91	CCAGGGCTATGAAACTGATGGA	CCAGGGCTATGAAACTGATGGA	
92	TTCTTACCCATTTAATTA	TTCTTACCCATTTAATTA	
93	TGCTTCTTACCCATTTAA	TGCTTCTTACCCATTTAA	
94	TAATGCTTCTTACCCATT	TAATGCTTCTTACCCATT	
95	AGATAATGCTTCTTACCC	AGATAATGCTTCTTACCC	
96	CAGATAATGCTTCTTACC	CAGATAATGCTTCTTACC	
97	CCCTTCAGATAATGCTTC	CCCTTCAGATAATGCTTC	
98	CTACTCCCTTCAGATAAT	CTACTCCCTTCAGATAAT	
99	AGCTCCTACTCCCTTCAG	AGCTCCTACTCCCTTCAG	
100	TTCACAGCTCCTACTCCC	TTCACAGCTCCTACTCCC	

FIG. 15 cont.

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Chemistry	
Compound ID No.:	Sequence (5'-3') (All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
101	TAAAATTACAGCTCCTA TAAAATCAGCTCCTA
102	AAATCTAAAATTCACAGC AAATCTAAAATTCACAGC
103	GAATAAAATCTAAAATTC GAATAAAATCTAAAATTC
104	GATGGGAATAAAAATCTAA GATGGGAATAAAAATCTAA
105	GCTGTGATGGGAATAAAA GCTGTGATGGGAATAAAA
106	TAGAGGCTGTGATGGGAA TAGAGGCTGTGATGGGAA
107	AAAGATAGAGGCTGTGAT AAAGATAGAGGCTGTGAT
108	AAAAGAAAGATAGAGGCT AAAAGAAAGATAGAGGCT
109	GACCTAAAAGAAAGATAG GACCTAAAAGAAAGATAG
110	ATAAAGACCTAAAAGAAA ATAAAGACCTAAAAGAAA
111	GAGATATAAAGACCTAAA GAGATATAAAGACCTAAA
112	GGCTGTGATGGGAATAAA GGCTGTGATGGGAATAAA
113	AGGCTGTGATGGGAATAA AGGCTGTGATGGGAATAA
114	GAGGCTGTGATGGGAATA GAGGCTGTGATGGGAATA
115	AGAGGCTGTGATGGGAAT AGAGGCTGTGATGGGAAT
116	ATAGAGGCTGTGATGGGA ATAGAGGCTGTGATGGGA
117	GATAGAGGCTGTGATGGG GATAGAGGCTGTGATGGG
118	AGATAGAGGCTGTGATGG AGATAGAGGCTGTGATGG
119	AAGATAGAGGCTGTGATG AAGATAGAGGCTGTGATG
120	TAGAGGCTGTGATGGGAATAA TAGAGGCTGTGATGGGAATAA

FIG. 15 cont.

Compound ID No.:	Sequence (5'-3')	Chemistry (All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
121	ATAGAGGCTGTGATGGGAATAA	ATAGAGGCTGTGATGGGAATAA
122	GATAGAGGCTGTGATGGGAATA	GATAGAGGCTGTGATGGGAATA
123	AGATAGAGGCTGTGATGGGAAT	AGATAGAGGCTGTGATGGGAAT
124	AAGATAGAGGCTGTGATGGGAA	AAGATAGAGGCTGTGATGGGAA
125	GAGGCTGTGATGGGAATAAA	GAGGCTGTGATGGGAATAAA
126	AGAGGCTGTGATGGGAATAA	AGAGGCTGTGATGGGAATAA
127	TAGAGGCTGTGATGGGAATA	TAGAGGCTGTGATGGGAATA
128	ATAGAGGCTGTGATGGGAAT	ATAGAGGCTGTGATGGGAAT
129	GATAGAGGCTGTGATGGGAA	GATAGAGGCTGTGATGGGAA
130	AGATAGAGGCTGTGATGGGA	AGATAGAGGCTGTGATGGGA
131	AAGATAGAGGCTGTGATGGG	AAGATAGAGGCTGTGATGGG
132	CTGTGATGGGAATAAA	CTGTGATGGGAATAAA
133	GCTGTGATGGGAATAA	GCTGTGATGGGAATAA
134	GGCTGTGATGGGAATA	GGCTGTGATGGGAATA
135	AGGCTGTGATGGGAAT	AGGCTGTGATGGGAAT
136	GAGGCTGTGATGGGAA	GAGGCTGTGATGGGAA
137	AGAGGCTGTGATGGGA	AGAGGCTGTGATGGGA
138	TAGAGGCTGTGATGGG	TAGAGGCTGTGATGGG
139	ATAGAGGCTGTGATGG	ATAGAGGCTGTGATGG
140	GATAGAGGCTGTGATG	GATAGAGGCTGTGATG

FIG. 15 cont.

Compound ID No.:	Sequence (5'-3')	Chemistry	
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
141	AGATAGAGGCTGTGAT	AGATAGAGGCTGTGAT	
142	AAGATAGAGGCTGTGA	AAGATAGAGGCTGTGA	
143	AGGCTGTGATGTGAATAA	AGGCTGTGATGTGAATAA	
144	AGGCTGTGATGTGAATAA	AGGCTGTGATGTGAATAA	
145	AGGCTGTGATGTGAATAA	AGGCTGTGATGTGAATAA	
146	AGAGGCTGTGATGTGAAT	AGAGGCTGTGATGTGAAT	
147	AGAGGCTGTGATGTGAAT	AGAGGCTGTGATGTGAAT	
148	AGAGGCTGTGATGTGAAT	AGAGGCTGTGATGTGAAT	
149	TAGAGGCTGTGATGTGAA	TAGAGGCTGTGATGTGAA	
150	TAGAGGCTGTGATGTGAA	TAGAGGCTGTGATGTGAA	
151	TAGAGGCTGTGATGTGAA	TAGAGGCTGTGATGTGAA	
152	GATAGAGGCTGTGATTGG	GATAGAGGCTGTGATTGG	
153	GATAGAGGCTGTGATTGG	GATAGAGGCTGTGATTGG	
154	GATAGAGGCTGTGATTGG	GATAGAGGCTGTGATTGG	
155	AGATAGAGGCTGTGATGG	AGATAGAGGCTGTGATGG	
156	GGCTGTGATGTGAATA	GGCTGTGATGTGAATA	
157	GGCTGTGATGTGAATA	GGCTGTGATGTGAATA	
158	GGCTGTGATGTGAATA	GGCTGTGATGTGAATA	
159	GAGGCTGTGATGTGAA	GAGGCTGTGATGTGAA	
160	GAGGCTGTGATGTGAA	GAGGCTGTGATGTGAA	

FIG. 15 cont.

Compound ID No.:		Sequence (5'-3')	Chemistry
			(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
161		GAGGCTGTGATGTGAA	GAGGCTGTGATGTGAA
162		TAGAGGCTGTGATTGG	TAGAGGCTGTGATTGG
163		TAGAGGCTGTGATTGG	TAGAGGCTGTGATTGG
164		TAGAGGCTGTGATTGG	TAGAGGCTGTGATTGG
165		ATAGAGGCTGTGATTGG	ATAGAGGCTGTGATTGG
166		GATAGAGGCTGTGATTGG	GATAGAGGCTGTGATTGG
167		AGATAGAGGCTGTGATTGG	AGATAGAGGCTGTGATTGG
168		GGCTATGAAACTGATGGAGA	GGCTATGAAACTGATGGAGA
169		CTATGAAACTGATGGAGA	CTATGAAACTGATGGAGA
170		GCTATGAAACTGATGGAG	GCTATGAAACTGATGGAG
171		GGCTATGAAACTGATGGAGA	GGCTATGAAACTGATGGAGA
172		ATGAAACTGATGGAGA	ATGAAACTGATGGAGA
173		ATGAAACTGATGGAGA	ATGAAACTGATGGAGA
174		CTATGAAACTGATGGAGA	CTATGAAACTGATGGAGA
175		CTATGAAACTGATGGAGA	CTATGAAACTGATGGAGA
176		GGCTATGAAACTGATGGAGA	GGCTATGAAACTGATGGAGA
177		GGCTATGAAACTGATGGAGA	GGCTATGAAACTGATGGAGA
178		GAAACTGATGGAGA	GAAACTGATGGAGA
179		ATGAAACTGATGGAGA	ATGAAACTGATGGAGA
180		CTATGAAACTGATGGAGA	CTATGAAACTGATGGAGA

FIG. 15 cont.

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Compound ID No.:	Sequence (5'-3')	Chemistry
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
181	GGCTATGAAACTGA	GGCTATGAAACTGA
182	TAGAGGCTGTGATGGGAATAAA	TAGAGGCTGTGATGGGAATAAA
183	TAGAGGCTGTGATGGGAATAAAAT	TAGAGGCTGTGATGGGAATAAAAT
184	ATAGAGGCTGTGATGGGAATAA	ATAGAGGCTGTGATGGGAATAA
185	ATAGAGGCTGTGATGGGAATAAAA	ATAGAGGCTGTGATGGGAATAAAA
186	ATAGAGGCTGTGATGGGAATAAAAT	ATAGAGGCTGTGATGGGAATAAAAT
187	AAAGATAGAGGCTGTGATGGGAATA	AAAGATAGAGGCTGTGATGGGAATA
188	GGCTATGAAACTGATGGAGAA	GGCTATGAAACTGATGGAGAA
189	GGCTATGAAACTGATGGAGAAA	GGCTATGAAACTGATGGAGAAA
190	GGCTATGAAACTGATGGAGAAAGA	GGCTATGAAACTGATGGAGAAAGA
191	AGGGCTATGAAACTGATGGAGAAAG	AGGGCTATGAAACTGATGGAGAAAG
192	CATTTAATTAAATTATAT	CATTTAATTAAATTATAT
193	CCATTTAATTAAATTATA	CCATTTAATTAAATTATA
194	CCCATTTAATTAAATTAT	CCCATTTAATTAAATTAT
195	ACCCATTTAATTAAATTA	ACCCATTTAATTAAATTA
196	TACCCATTTAATTAAATT	TACCCATTTAATTAAATT
197	TTACCCATTTAATTAAAT	TTACCCATTTAATTAAAT
198	CTTACCCATTTAATTAAA	CTTACCCATTTAATTAAA
199	TCTTACCCATTTAATTAA	TCTTACCCATTTAATTAA
200	GATAGAGGCTGTGATGG	GATAGAGGCTGTGATGG

FIG. 15 cont.

Compound ID No.:	Sequence (5'-3')	Chemistry
201	GGCTGTGAAACTGATGGA	GGCTGTGAAACTGATGGA
202	GGCTGTGAAACTGATGGAGA	GGCTGTGAAACTGATGGAGA
203	CTATGAAACTGATGGA	CTATGAAACTGATGGA
204	GCTATGAAACTGATGG	GCTATGAAACTGATGG
205	GGCTATGAAACTGATG	GGCTATGAAACTGATG
206	ATGAAACTGATGGA	ATGAAACTGATGGA
207	TATGAAACTGATGG	TATGAAACTGATGG
208	CTATGAAACTGATG	CTATGAAACTGATG
209	GCTATGAAACTGAT	GCTATGAAACTGAT
210	GGCTATGAAACTGA	GGCTATGAAACTGA
211	GCTGTGAAACTGATGGAGAA	GCTGTGAAACTGATGGAGAA
212	GGGCTGTGAAACTGATGGAG	GGGCTGTGAAACTGATGGAG
213	TGTGAAACTGATGGAGAA	TGTGAAACTGATGGAGAA
214	CTGTGAAACTGATGGAGA	CTGTGAAACTGATGGAGA
215	GCTGTGAAACTGATGGAG	GCTGTGAAACTGATGGAG
216	GGGCTGTGAAACTGATGG	GGGCTGTGAAACTGATGG
217	TGAAACTGATGGAGAA	TGAAACTGATGGAGAA
218	GTGAAACTGATGGAGA	GTGAAACTGATGGAGA
219	TGTGAAACTGATGGAG	TGTGAAACTGATGGAG
220	CTGTGAAACTGATGGA	CTGTGAAACTGATGGA

FIG. 15 cont.

Compound ID No.:		Sequence (5'-3')	Chemistry
			(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)
221	GCTGTGAAACTGATGG	GGTGTGAAACTGATGG	
222	GGCTGTGAAACTGATG	GGTGTGAAACTGATG	
223	GGGCTGTGAAACTGAT	GGGCTGTGAAACTGAT	
224	CGGTCCAGGAATGAC	CGGTCCAGGAATGAC	
225	CCGGTCCAGGAATGA	CCGGTCCAGGAATGA	
226	CCCGGTCCAGGAATG	CCCGGTCCAGGAATG	
227	TCCCGGTCCAGGAAT	TCCCGGTCCAGGAAT	
228	CTCCCGGTCCAGGAA	CTCCCGGTCCAGGAA	
229	GCTCCCGGTCCAGGA	GCTCCCGGTCCAGGA	
230	GGTCCCGGTCCAGG	GGTCCCGGTCCAGG	
231	CGGGAGCCCCCGTGT	CGGGAGCCCCCGTGT	
232	GCGGGAGCCCCCGTGT	GCGGGAGCCCCCGTGT	
233	CGCGGGAGCCCCCGGT	CGCGGGAGCCCCCGGT	
234	ACGCGGGAGCCCCCG	ACGCGGGAGCCCCCG	
235	CACGCGGGAGCCCCC	CACGCGGGAGCCCCC	
236	CCACGCGGGAGCCCC	CCACGCGGGAGCCCC	
237	GCCACGCGGGAGCCC	GCCACGCGGGAGCCC	
238	GGCCACGCGGGAGCC	GGCCACGCGGGAGCC	
239	CGGCCACGCGGGAGC	CGGCCACGCGGGAGC	
240	ACGGCCACGCGGGAG	ACGGCCACGCGGGAG	

FIG. 15 cont.

Compound ID No.:	Sequence (5'-3')	Chemistry	
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
241	GACGGCCACGCGGA	GACGGCCACGCGGA	
242	AGACGGCCACGCGGG	AGACGGCCACGCGGG	
243	GCTAGGGAGGGATGGTTA	GCTAGGGAGGGATGGTTA	
244	TGTAAGCTAGGGAGGGAT	TGTAAGCTAGGGAGGGAT	
245	ACAGATGTAAAGCTAGGGA	ACAGATGTAAAGCTAGGGA	
246	AAGGAACAGATGTAAGCT	AAGGAACAGATGTAAGCT	
247	CAACAAAGGAACAGATGT	CAACAAAGGAACAGATGT	
248	GGGTGCAACAAAGGAACA	GGGTGCAACAAAGGAACA	
249	ACCAAGGTGCAACAAAG	ACCAAGGTGCAACAAAG	
250	GTTAAACCAAGGTGCAA	GTTAAACCAAGGTGCAA	
251	ATAATGTAAACCAAGGG	ATAATGTAAACCAAGGG	
252	GGAGAAATAATGTTAAACC	GGAGAAATAATGTTAAACC	
253	GGGAGGAGAAATAATGTT	GGGAGGAGAAATAATGTT	
254	AAATTGGGGAGGAGAATA	AAATTGGGGAGGAGAATA	
255	AGAGGAAATTGGGGAGGA	AGAGGAAATTGGGGAGGA	
256	GGAGAAAGGAAATTGGG	GGAGAAAGGAAATTGGG	
257	AATGAGGAGAAAGAGGAAA	AATGAGGAGAAAGAGGAAA	
258	TTCACAAATGAGGAGAAGA	TTCACAAATGAGGAGAAGA	
259	ACGAGTTCACAAATGAGGA	ACGAGTTCACAAATGAGGA	
260	CTGCCACGAGTTCACAAT	CTGCCACGAGTTCACAAT	

FIG. 15 cont.

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Compound ID No.:	Sequence (5'-3')	Chemistry	
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
261	AGACCCCTGCCACGAGTTC	AGACCCCTGCCACGAGTTC	
262	CAAGCAGACCCTGCCACG	CAAGCAGACCCTGCCACG	
263	CTCACCACGACAGCCCTG	CTCACCACGACAGCCCTG	
264	GAGCTCACCACGACAGCC	GAGCTCACCACGACAGCC	
265	AGAATGAGCTCACCACG	AGAATGAGCTCACCACG	
266	GTAAGAGAATGAGCTCAC	GTAAGAGAATGAGCTCAC	
267	TTGTTGTAAGAGAATGAG	TTGTTGTAAGAGAATGAG	
268	ATTTGTTGTTGTAAGAGA	ATTTGTTGTTGTAAGAGA	
269	CTTGAATTTGTTGTTGTA	CTTGAATTTGTTGTTGTA	
270	ATGCTCTTGAATTTGTTG	ATGCTCTTGAATTTGTTG	
271	TCTTCATGCTCTTGAATT	TCTTCATGCTCTTGAATT	
272	CTTCCTCTTCATGCTCTT	CTTCCTCTTCATGCTCTT	
273	GCGCGCTTCCTCTTCATG	GCGCGCTTCCTCTTCATG	
274	GCTCTGCGCGCTTCCTCT	GCTCTGCGCGCTTCCTCT	
275	GGCCAGCGGCTCTGCGCG	GGCCAGCGGCTCTGCGCG	
276	ATATTGGCCAGCGGCTCT	ATATTGGCCAGCGGCTCT	
277	GTGCTATATTGGCCAGCG	GTGCTATATTGGCCAGCG	
278	AGCTCGTGCTATATTGGC	AGCTCGTGCTATATTGGC	
279	GGCATAGCTCGTGCTATA	GGCATAGCTCGTGCTATA	
280	TGTTGGGCATAGCTCGTG	TGTTGGGCATAGCTCGTG	

FIG. 15 cont.

		Chemistry	
Compound ID No.:	Sequence (5'-3')	(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
281	GCTTCTGTGGGCATAGC	GCTTCTGTGGGCATAGC	
282	CTTGGCTTCTGTGGGC	CTTGGCTTCTGTGGGC	
283	CACCTTGGCTTCTGTG	CACCTTGGCTTCTGTG	
284	TCCATCACCTTGGGCTTC	TCCATCACCTTGGGCTTC	
285	AACCATCCATCACCTTGC	AACCATCCATCACCTTGC	
286	CCTTAAACCATCCATCAC	CCTTAAACCATCCATCAC	
287	AGCCCCCTTAAACCATCC	AGCCCCCTTAAACCATCC	
288	TCGGTAGCCCCCTTAAAC	TCGGTAGCCCCCTTAAAC	
289	ATGTATCGGTAGCCCCCT	ATGTATCGGTAGCCCCCT	
290	TGTGAATGTATCGGTAGC	TGTGAATGTATCGGTAGC	
291	ATTAGTGTGAATGTATCG	ATTAGTGTGAATGTATCG	
292	GGCTGATTAGTGTGAATG	GGCTGATTAGTGTGAATG	
293	GAAATGGCTGATTAGTGT	GAAATGGCTGATTAGTGT	
294	TGGCAGAAATGGCTGATT	TGGCAGAAATGGCTGATT	
295	GATCTTGGCAGAAATGGC	GATCTTGGCAGAAATGGC	
296	GACATGATCTTGGCAGAA	GACATGATCTTGGCAGAA	
297	GAGGTGACATGATCTTGG	GAGGTGACATGATCTTGG	
298	AGATTGAGGTGACATGAT	AGATTGAGGTGACATGAT	
299	TGAACAGATTGAGGTGAC	TGAACAGATTGAGGTGAC	
300	GTCCATGAACAGATTGAG	GTCCATGAACAGATTGAG	

FIG. 15 cont.

Compound ID No.:		Sequence (5'-3')	Chemistry
		(All MOE unless otherwise noted; G-LNA, C-5MeC-MOE)	
301		TTGGAGTCCATGAACAGA	TTGGAGTCCATGAACAGA
302		TGTATTGGAGTCCATGA	TGTATTGGAGTCCATGA
303		TTTCTTGTATTGGAGTC	TTTCTTGTATTGGAGTC

FIG. 15 cont.

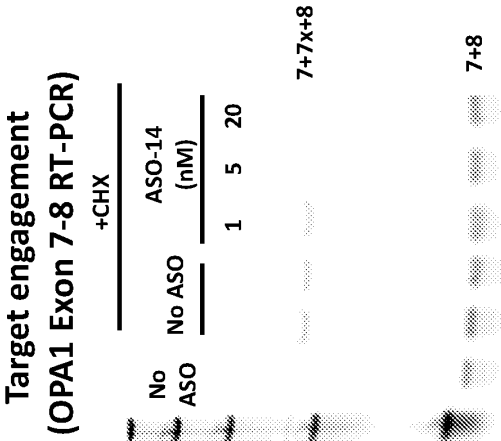


FIG. 16A

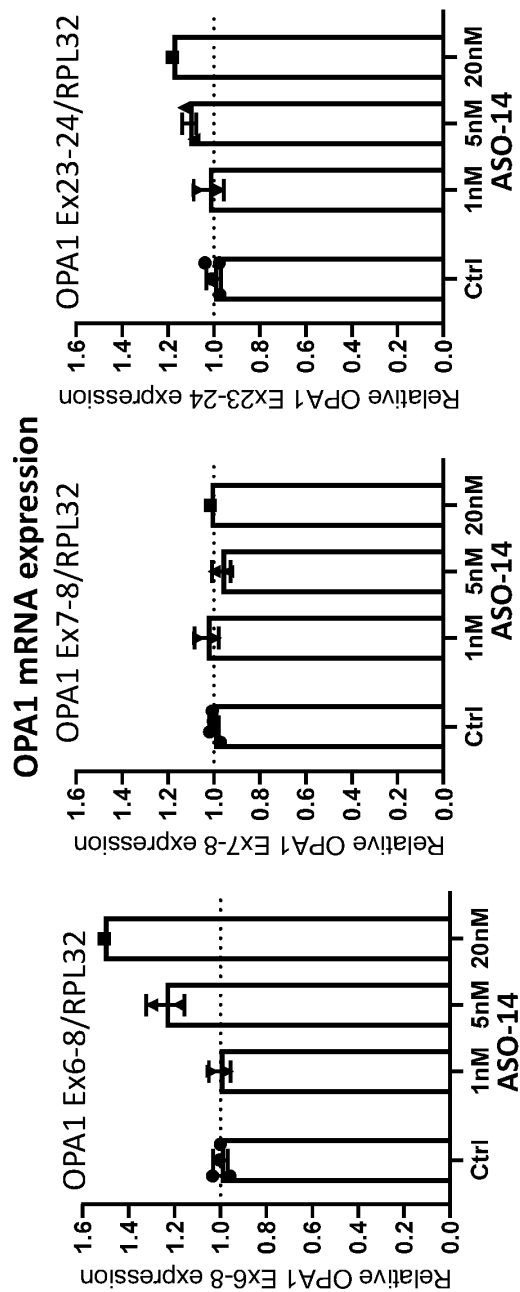


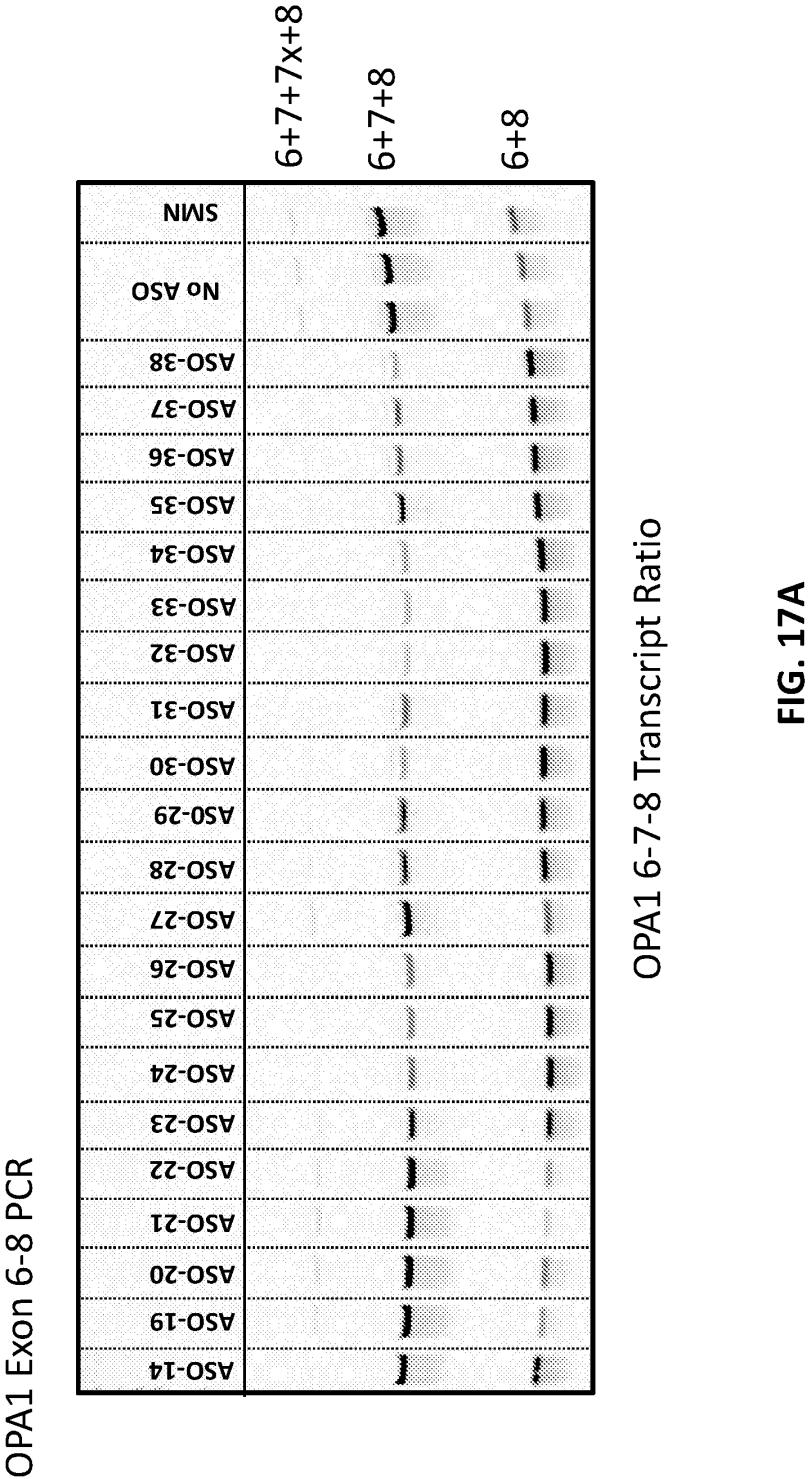
FIG. 16B

MinION Sequencing Data in HEK293 cells transfected with ASO-14

	Isoform	Exons	Mock	ASO-14
1		6-7-8	60.1%	49.0%
2		6-8	35.8%	47.3%
3*		6a-8	1.2%	1.3%
4*		6-7x-8	n.d.	n.d.
5*		6-7-7x-8	0.6%	0.0%

FIG. 16C

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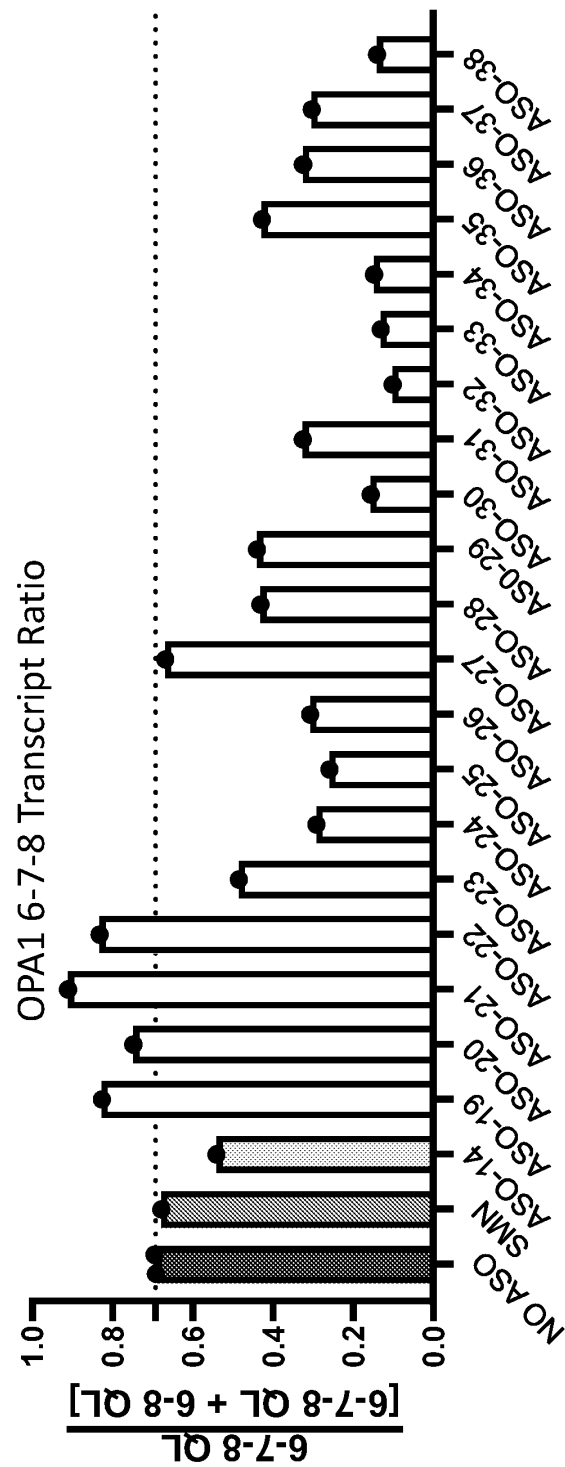


FIG. 17B

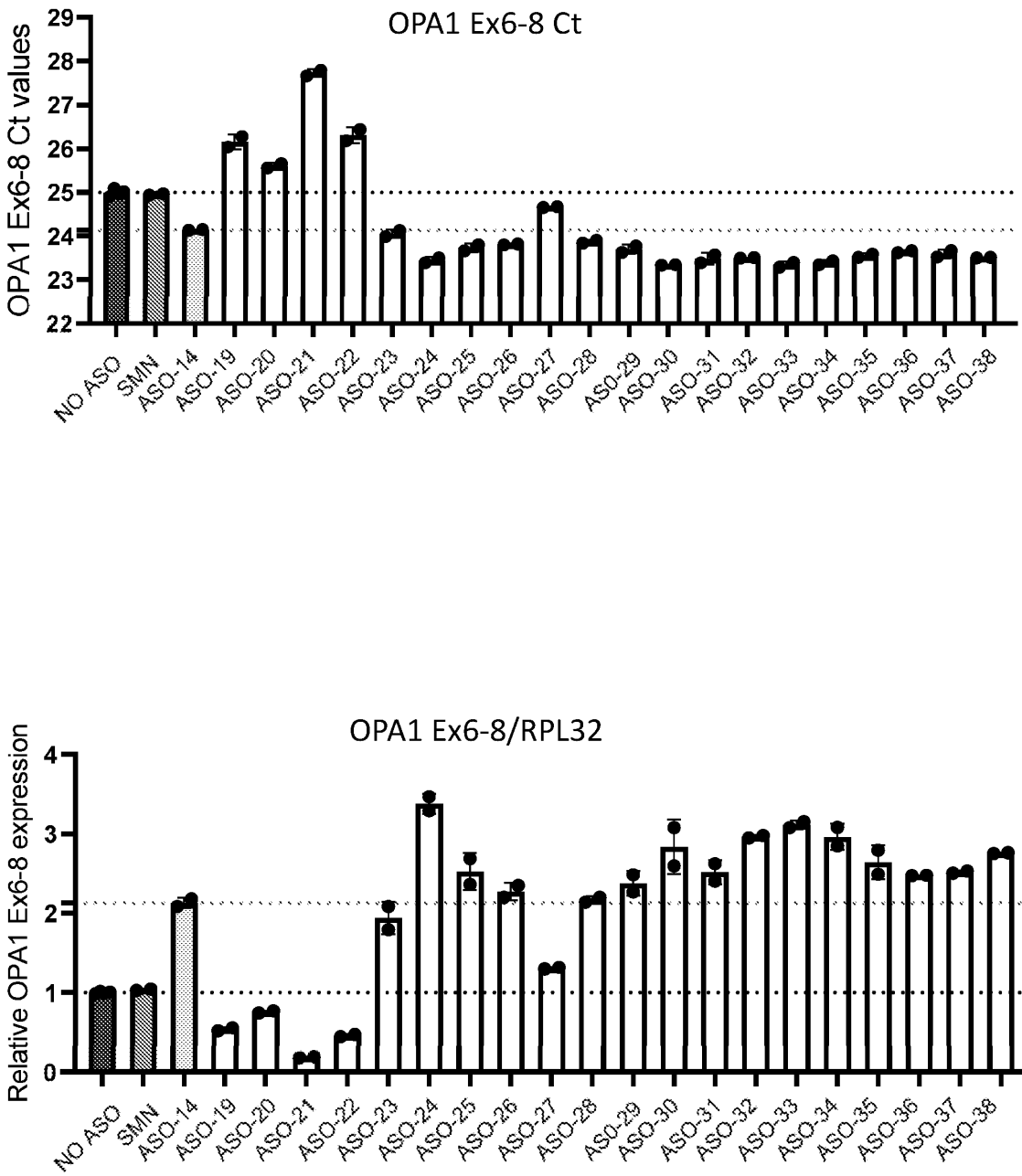


FIG. 17C

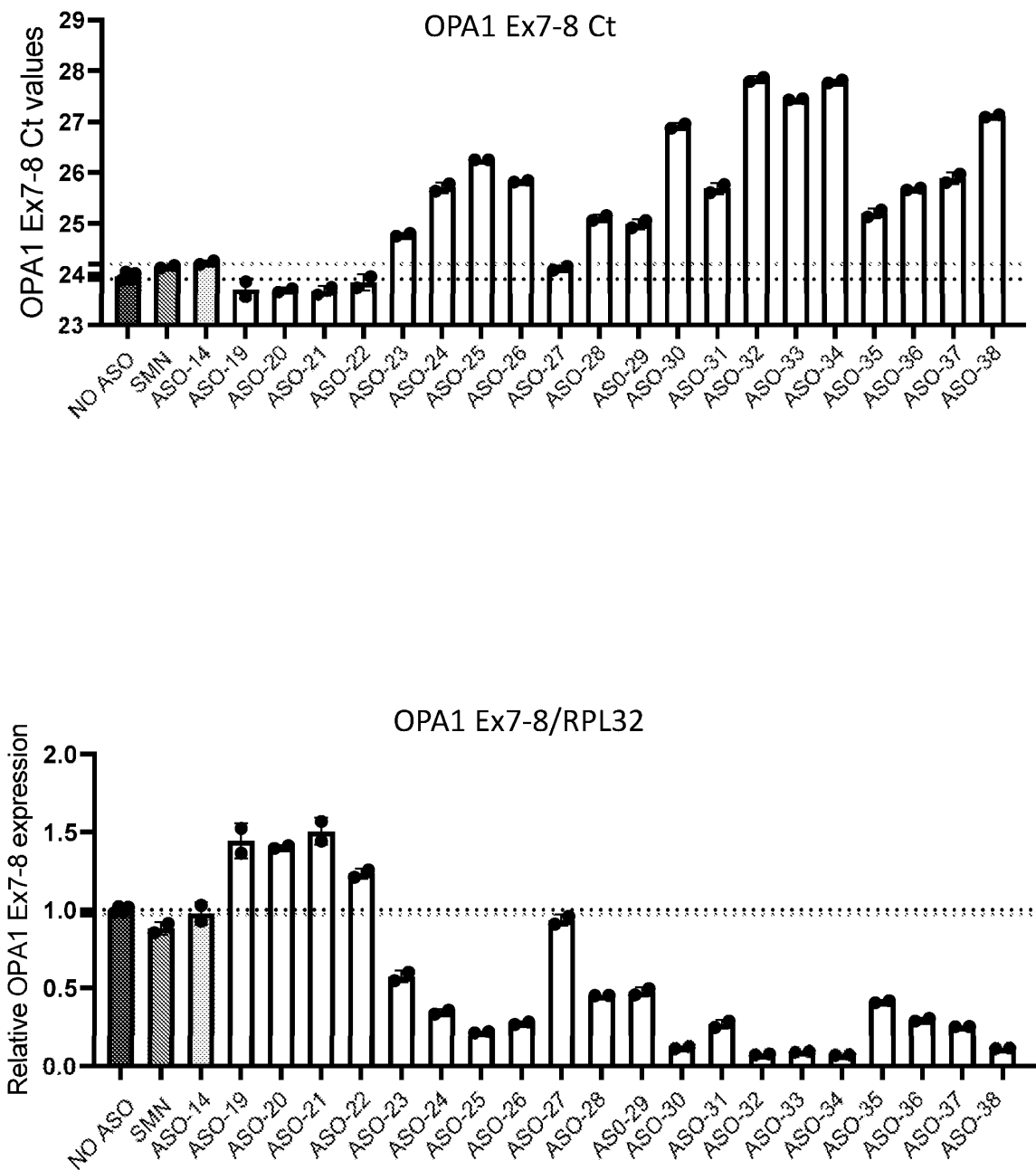


FIG. 17D

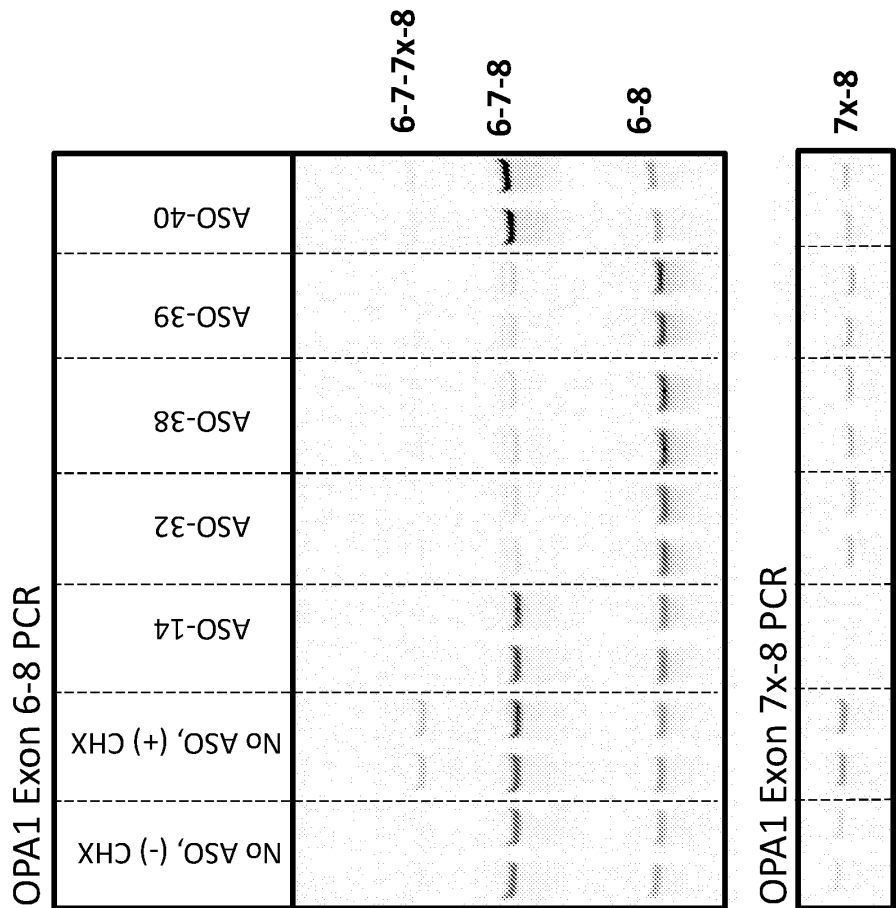


FIG. 18A

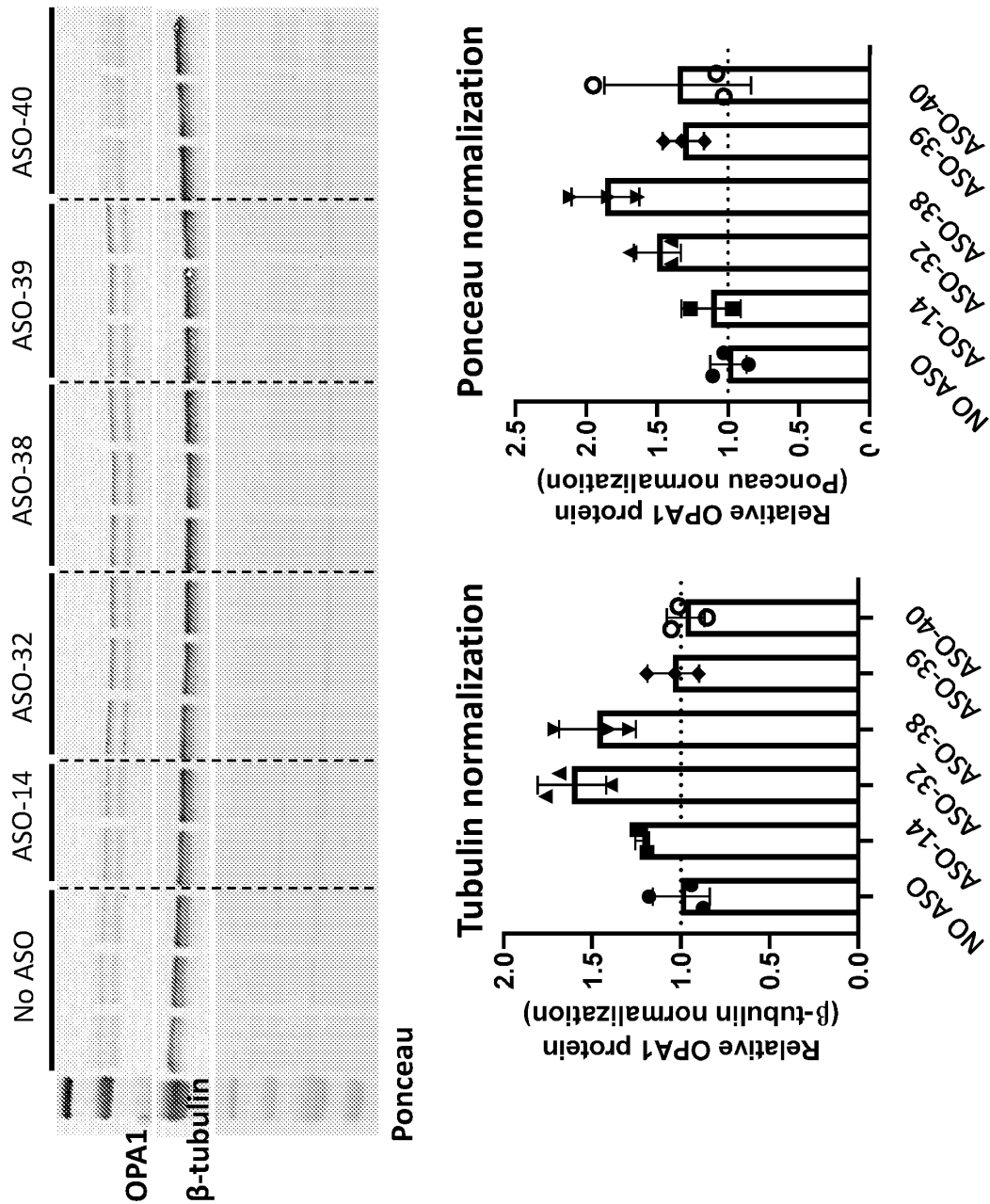


FIG. 18B

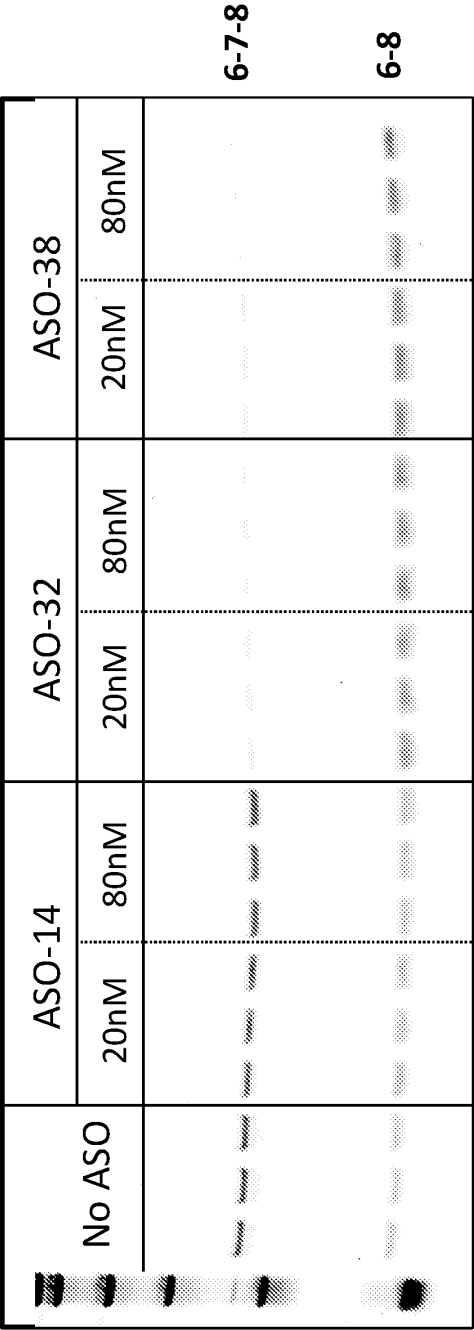


FIG. 18C

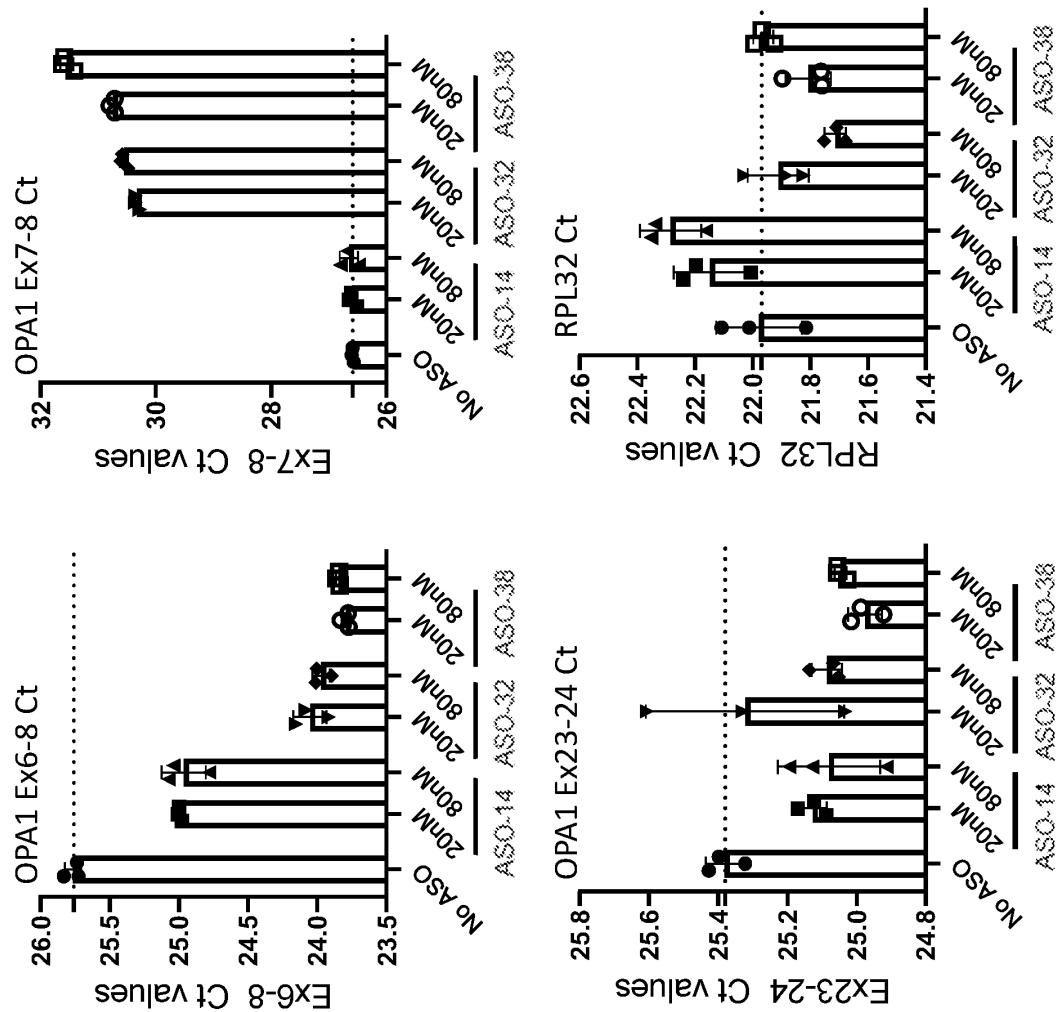


FIG. 18D

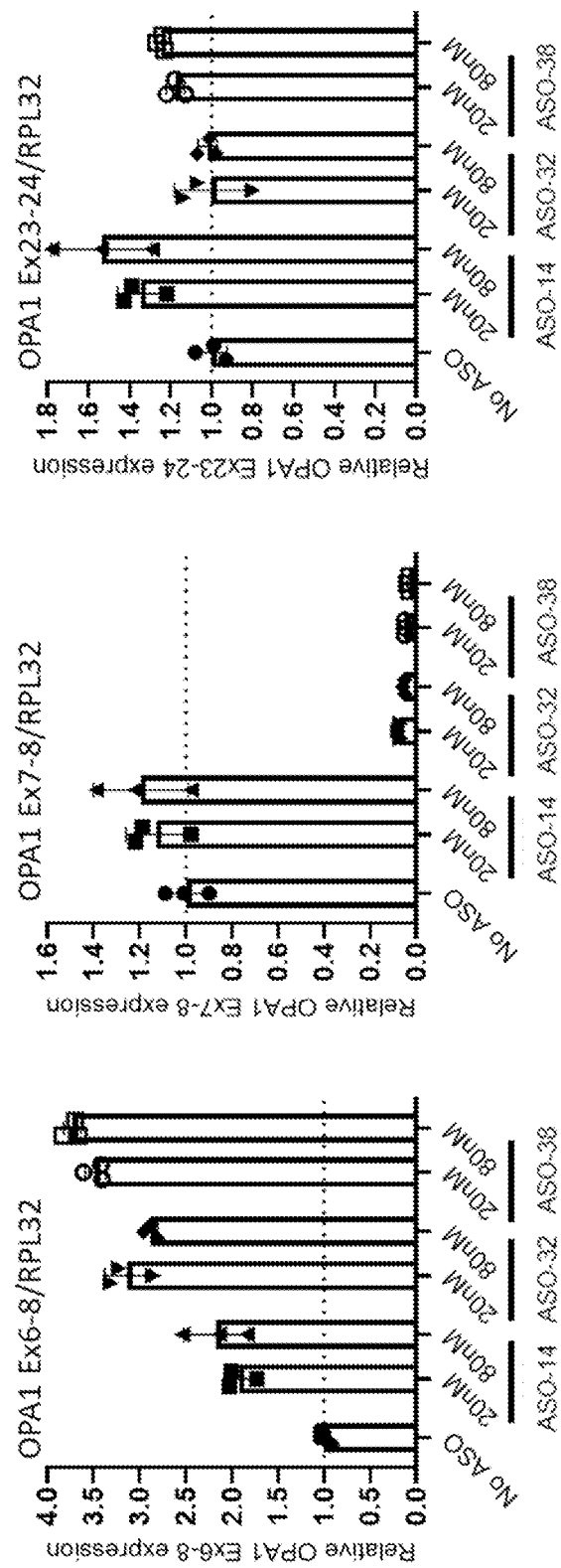


FIG. 18E

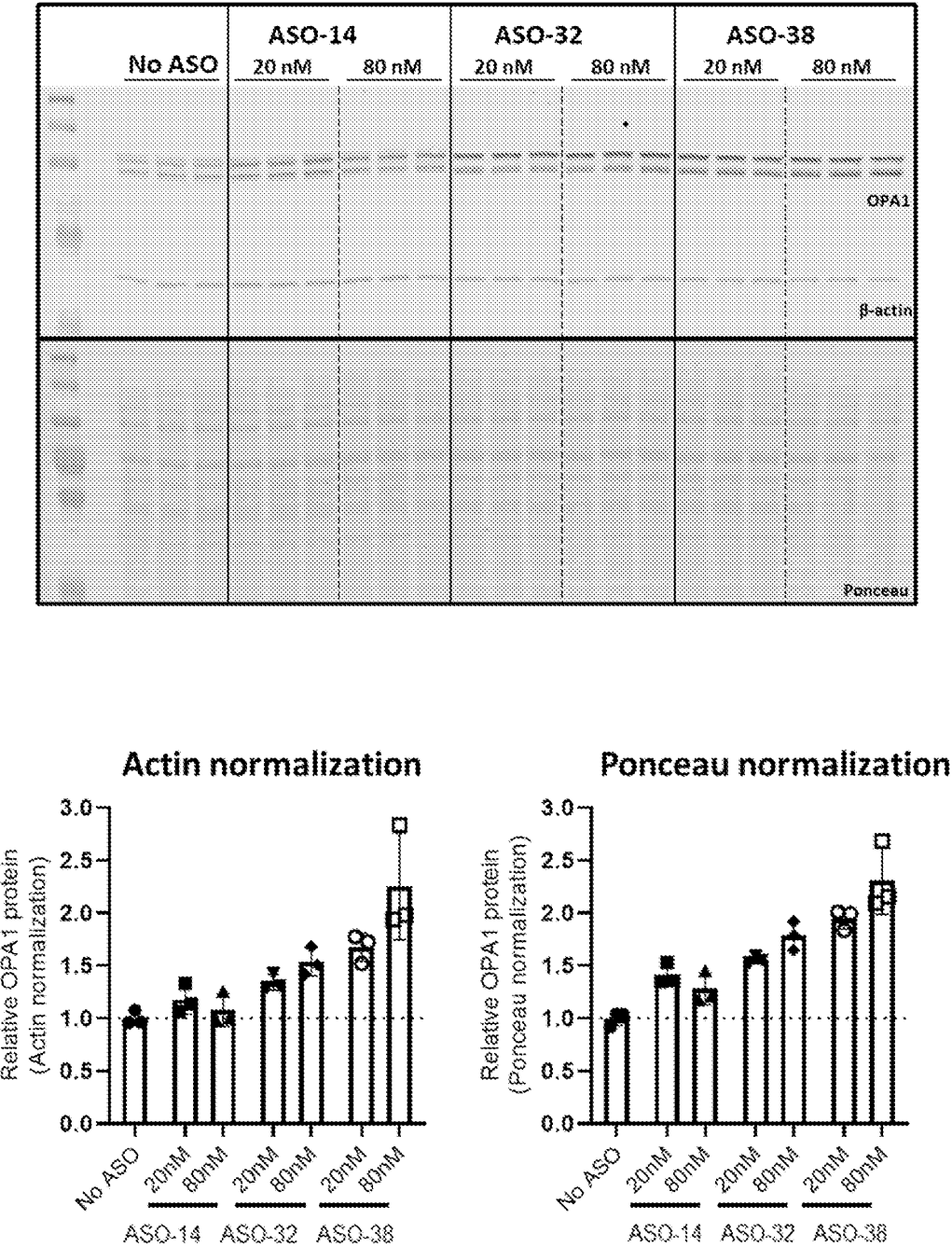


FIG. 18F

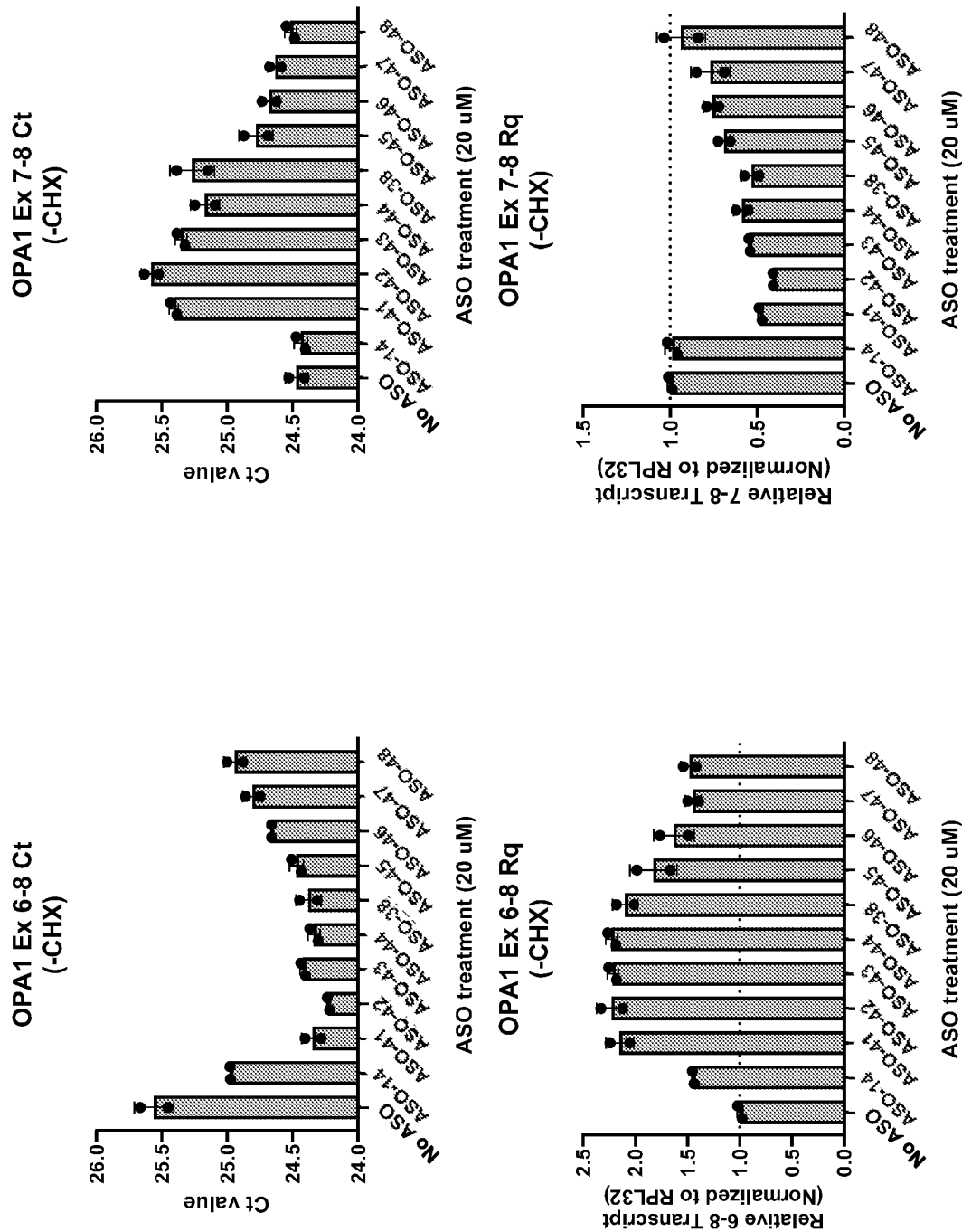
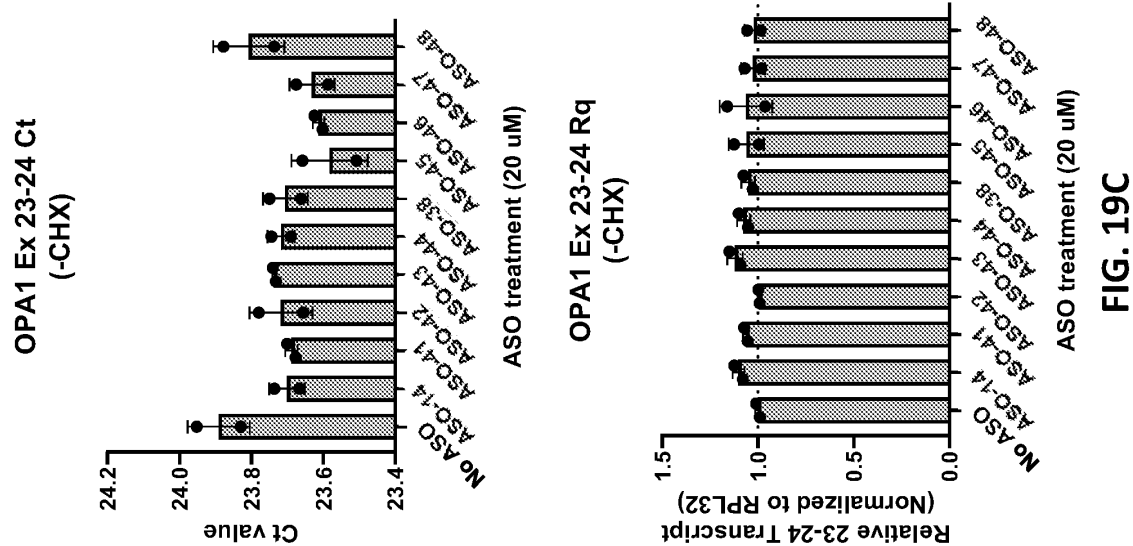
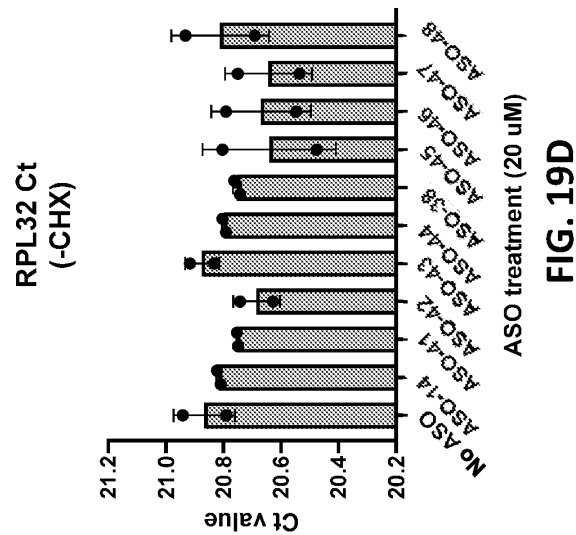


FIG. 19A

FIG. 19B



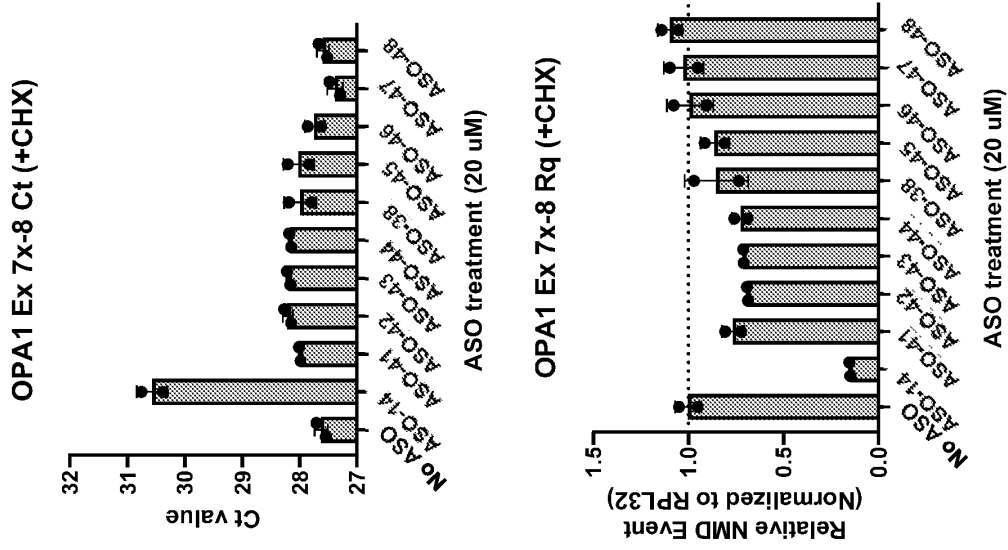


FIG. 20A

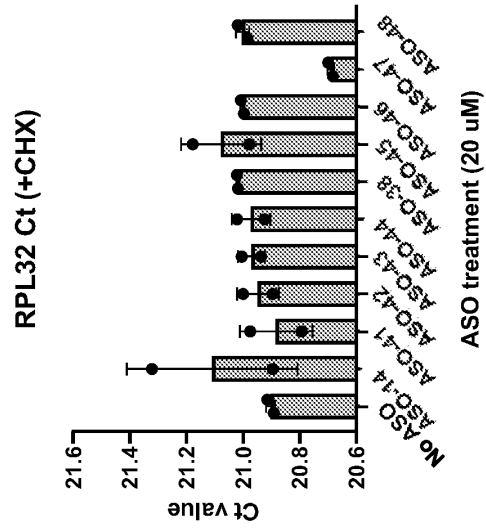


FIG. 20B

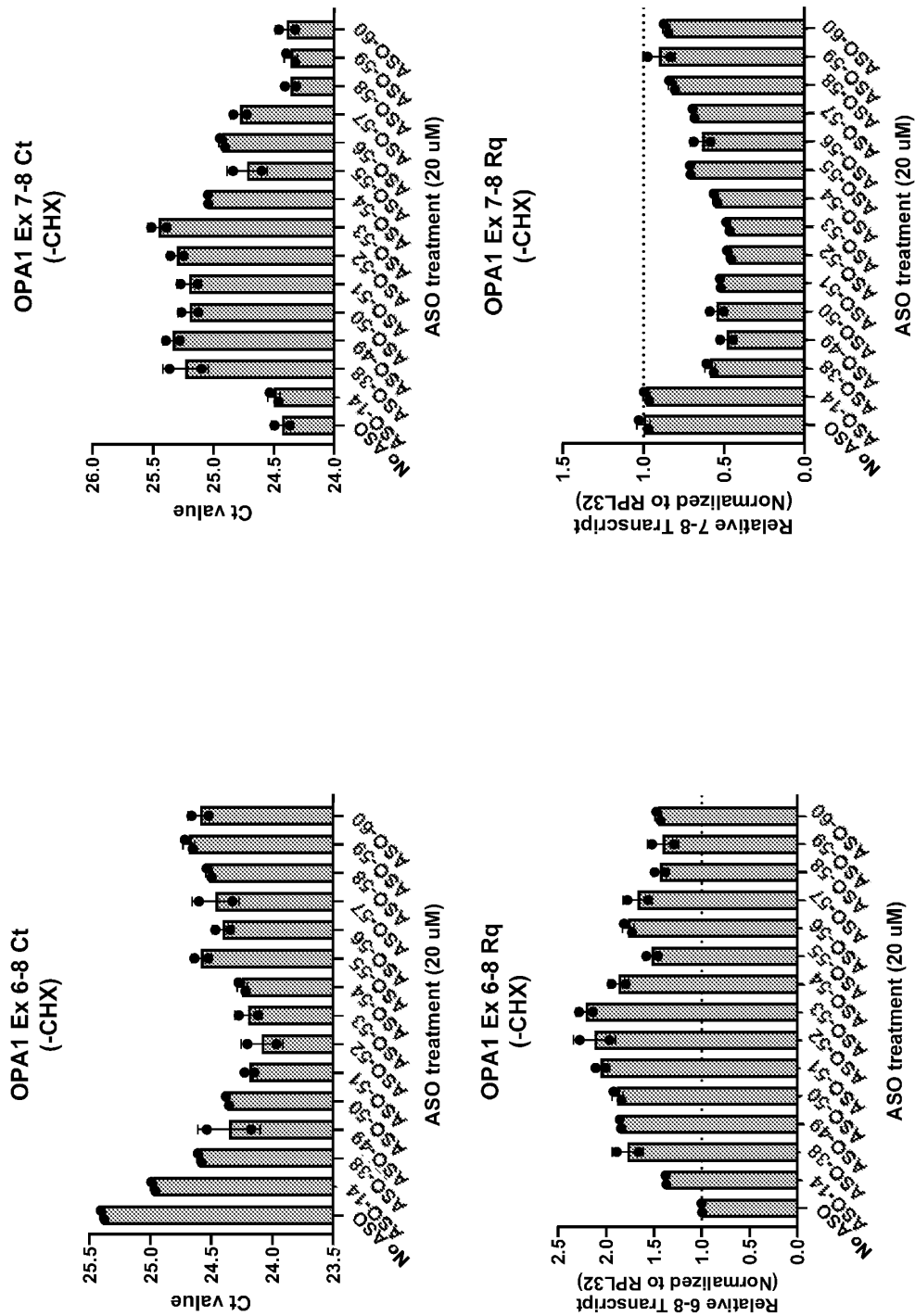


FIG. 21B

FIG. 21A

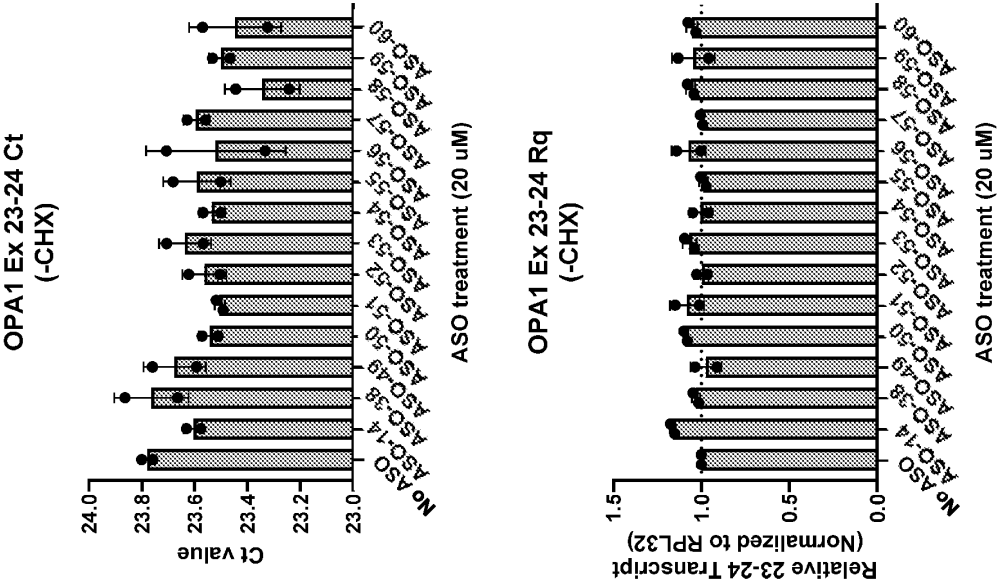


FIG.21C

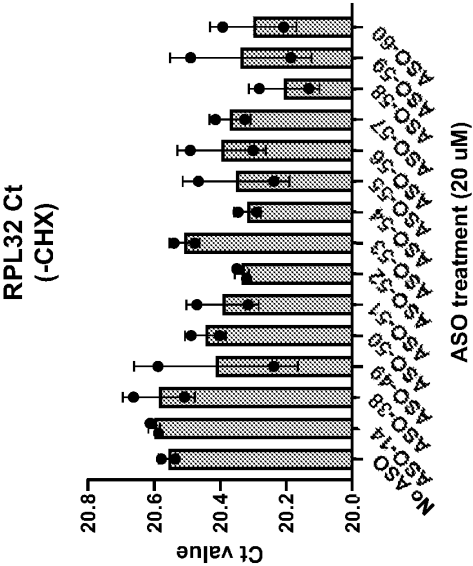
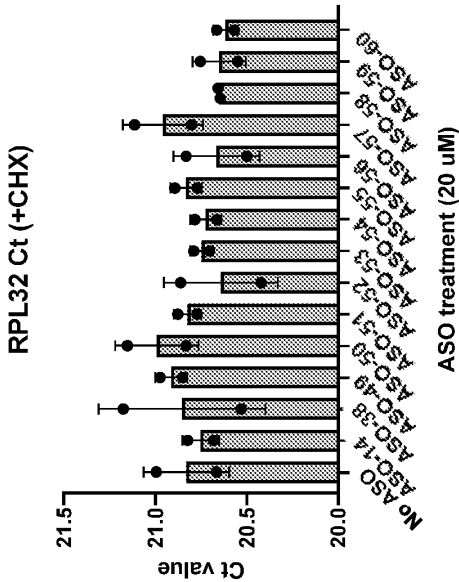
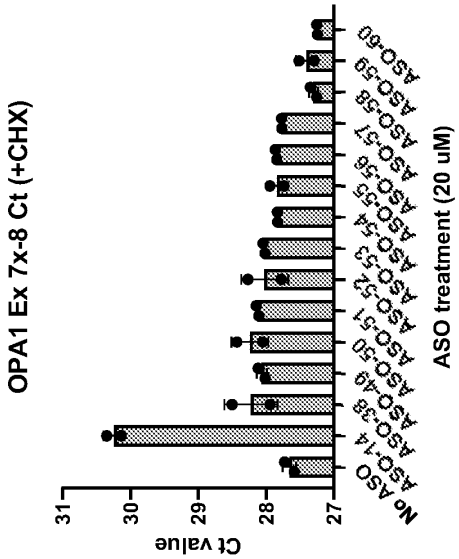


FIG.21D



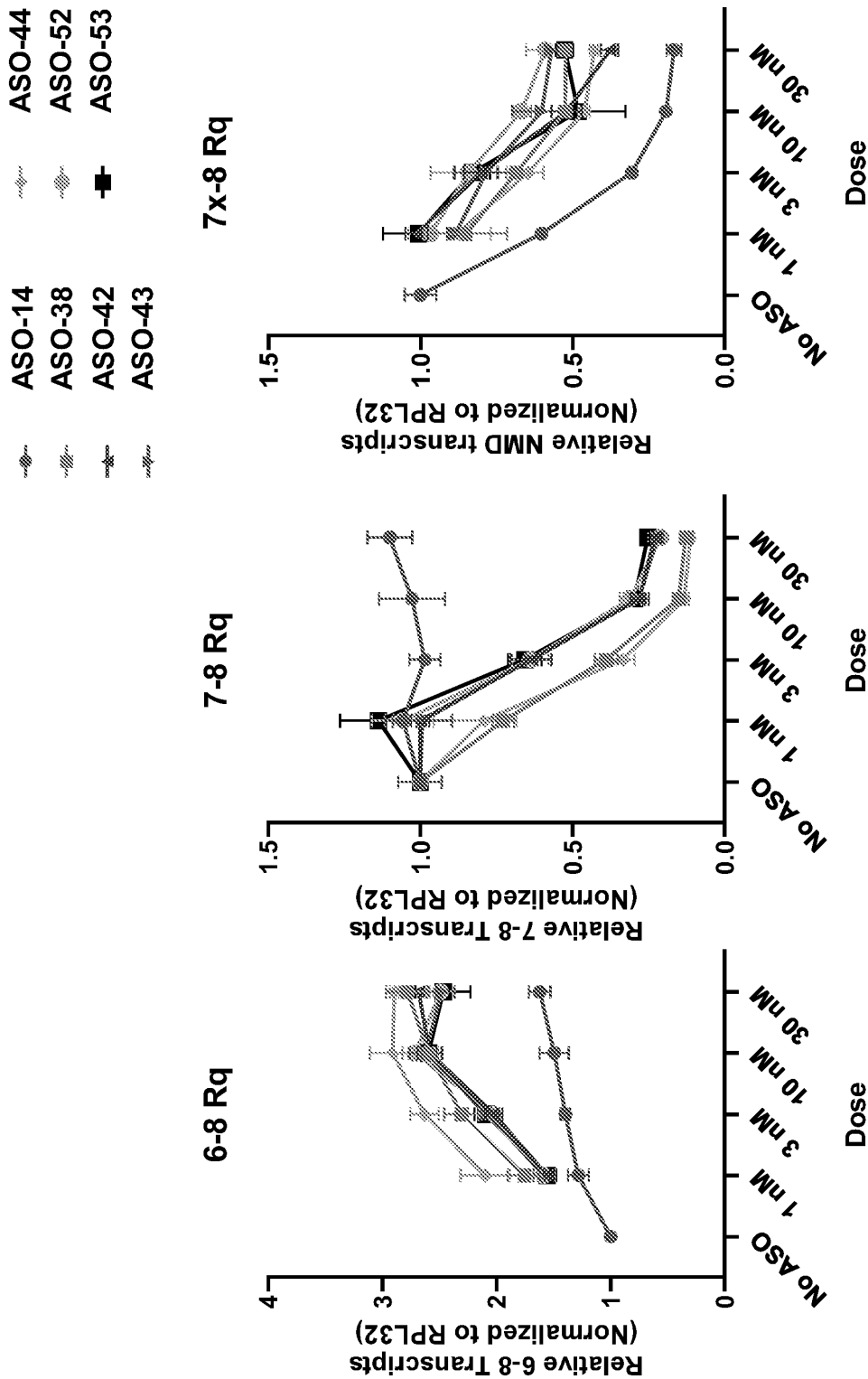


FIG.23A

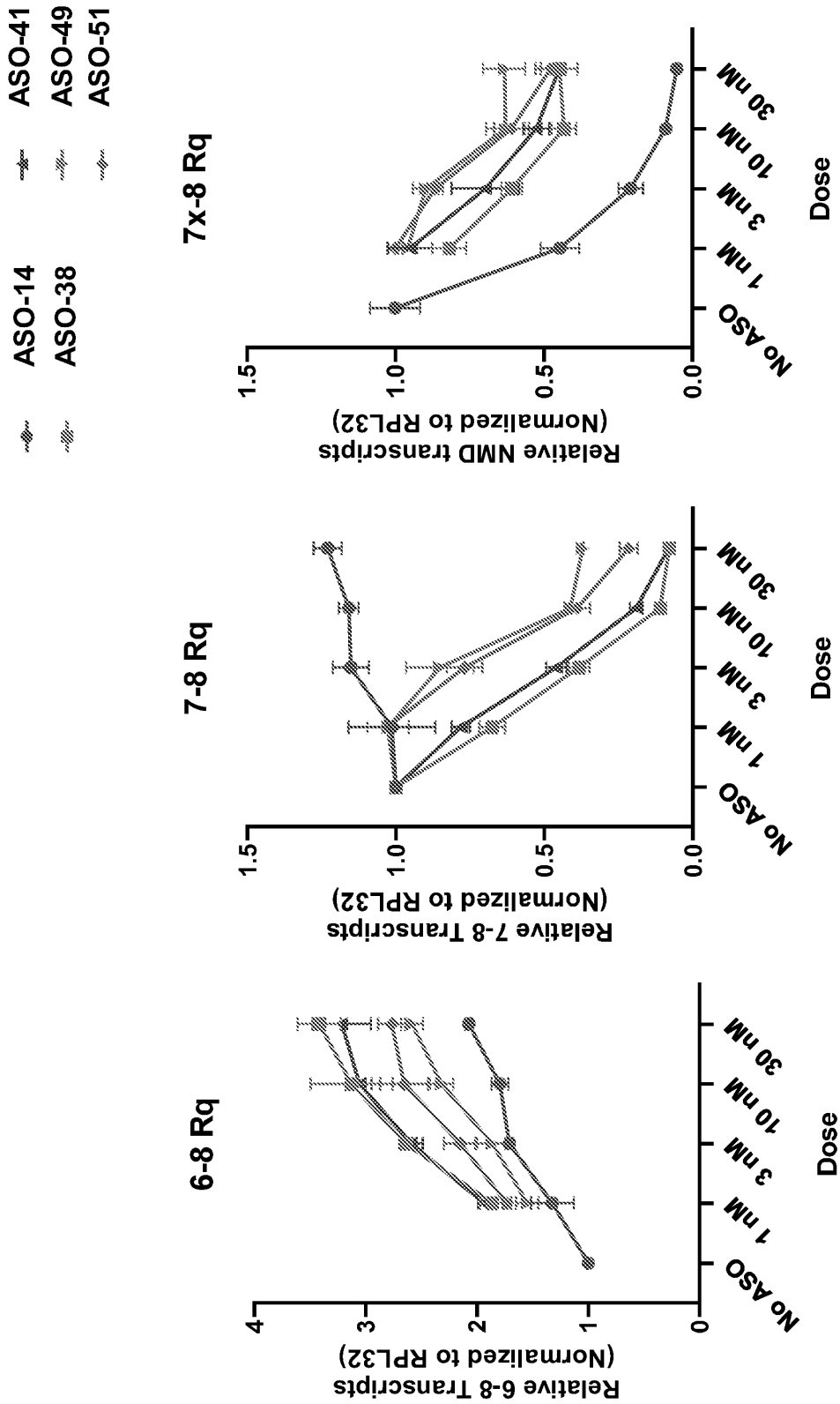
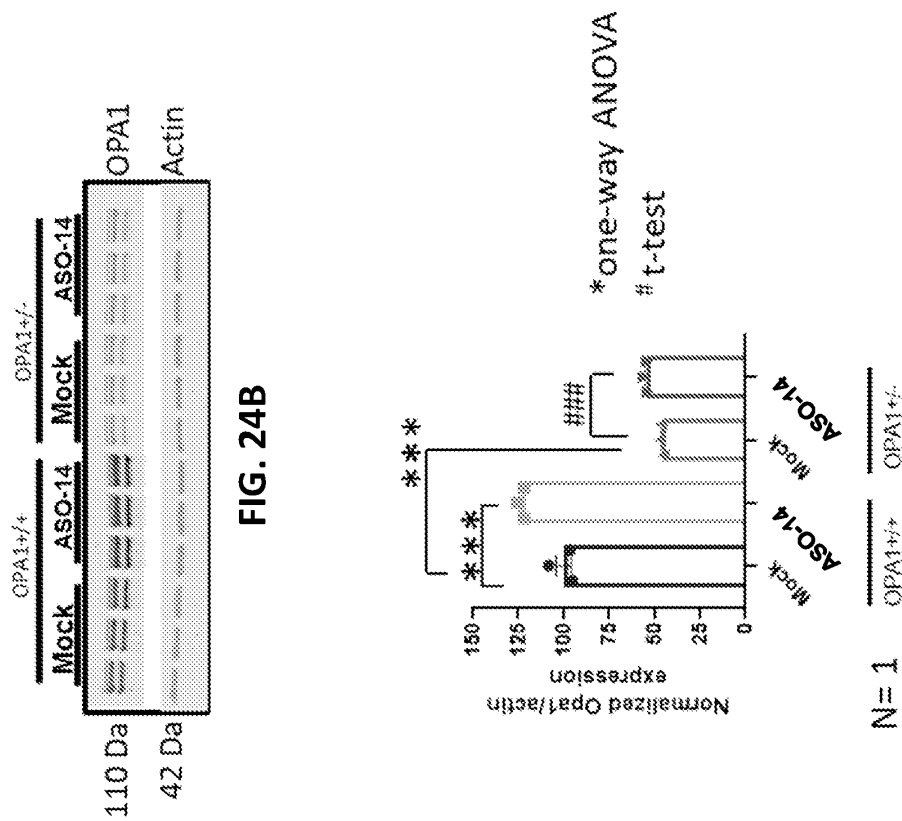
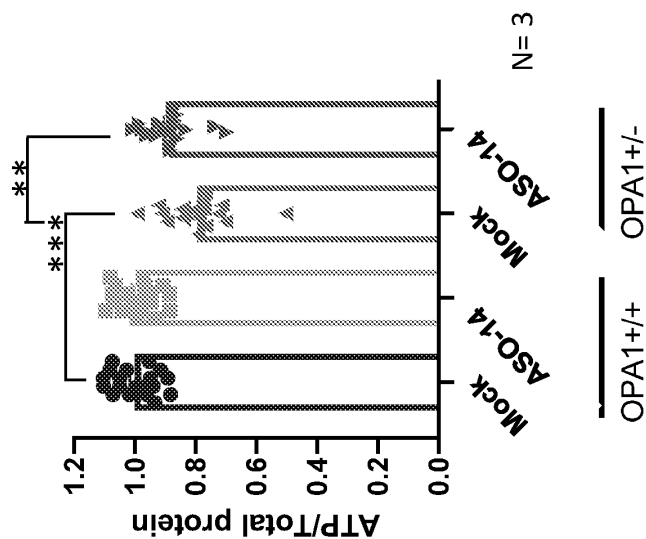


FIG.23B



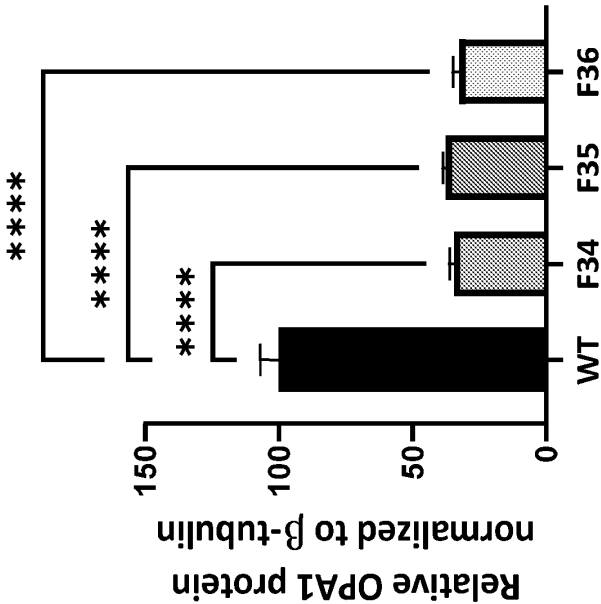


FIG. 25B

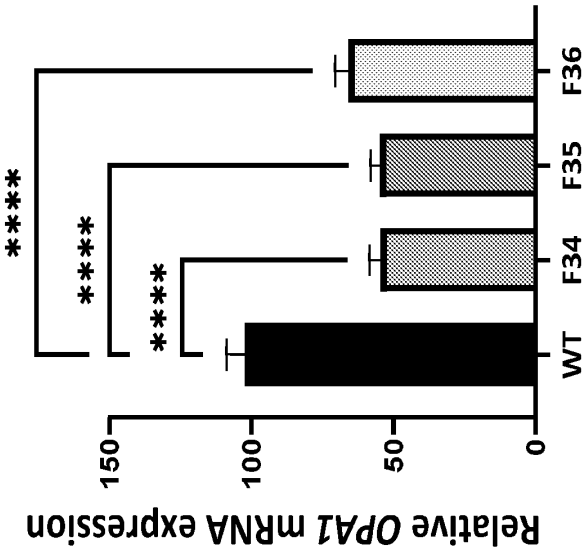


FIG. 25A

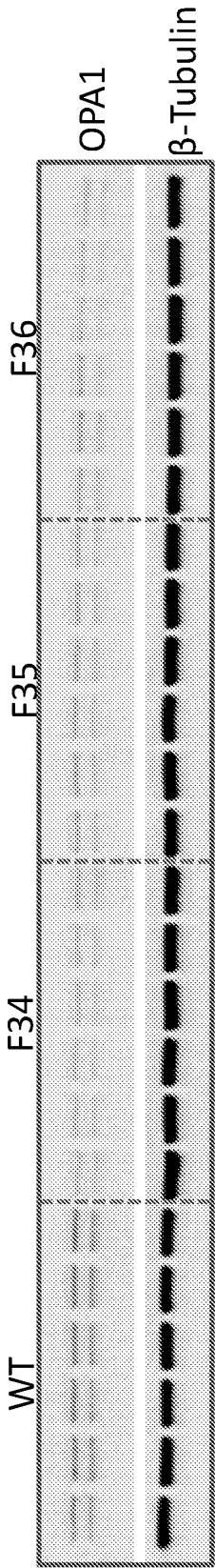


FIG. 25C

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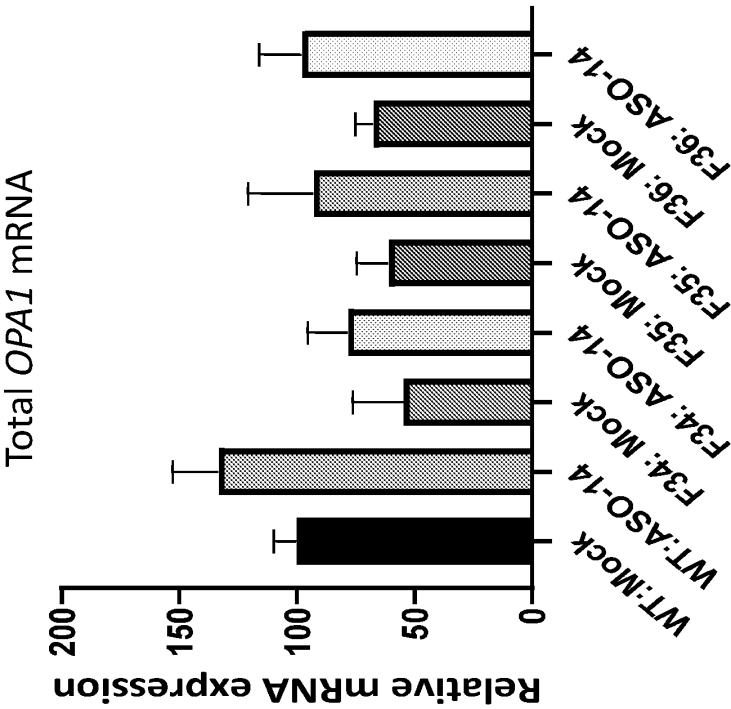


FIG. 26B

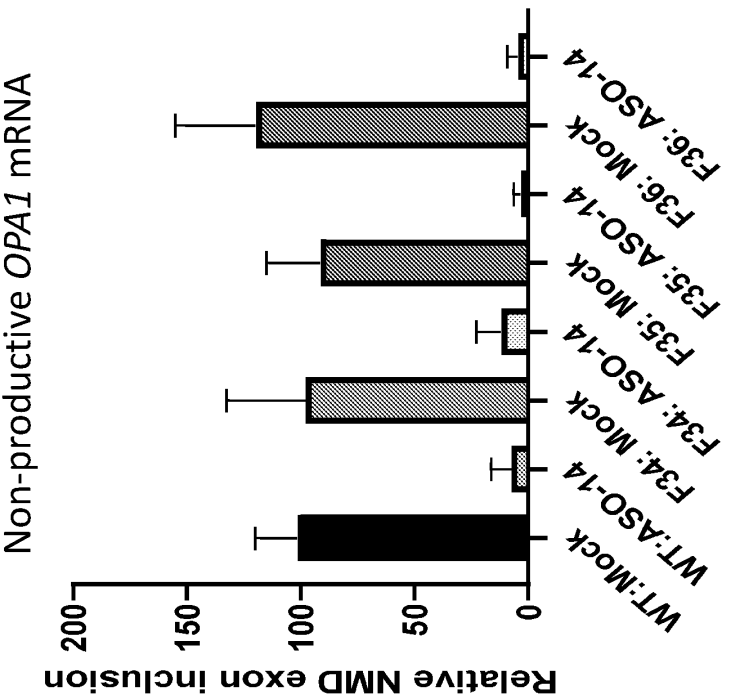


FIG. 26A

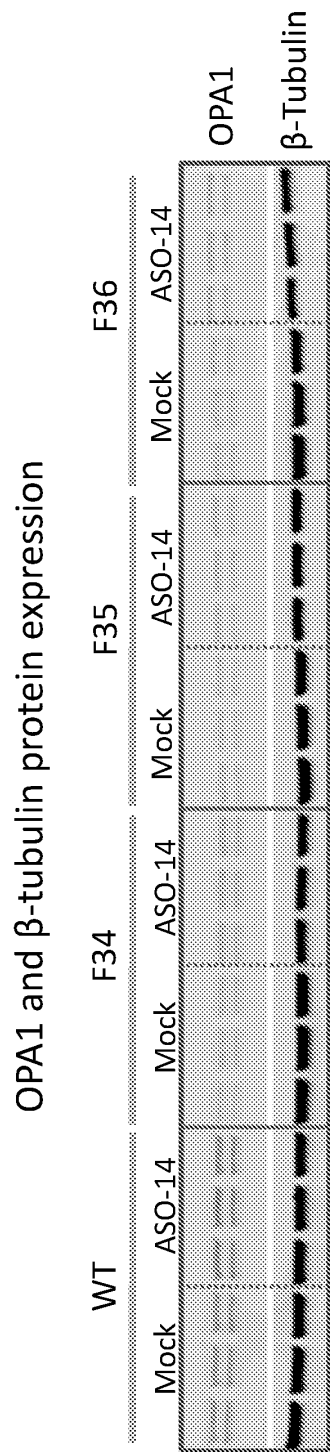


FIG. 26C

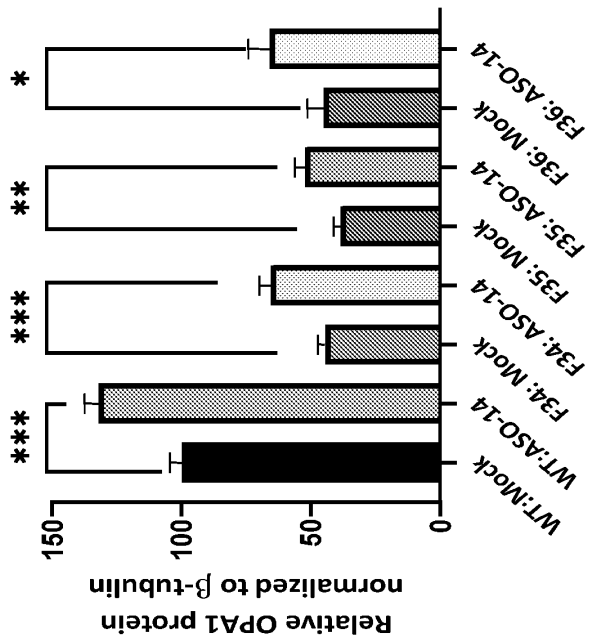
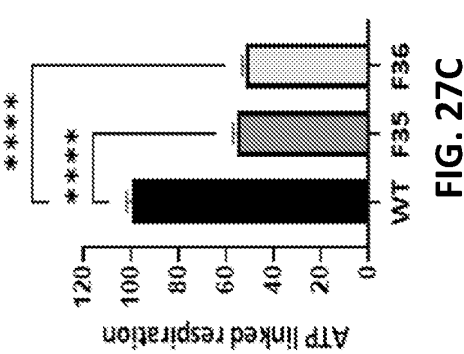
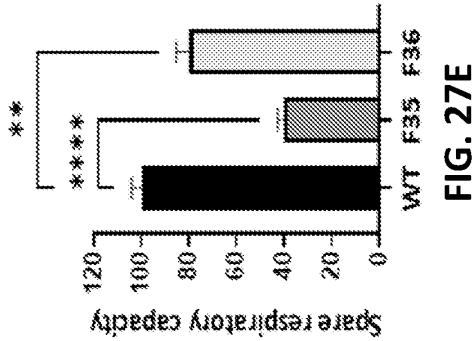
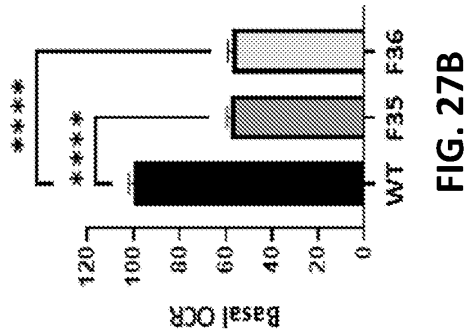
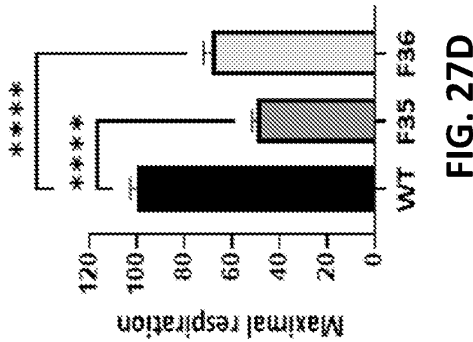
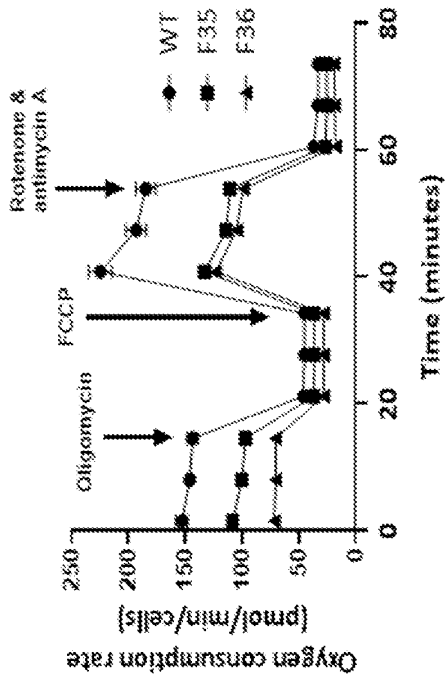


FIG. 26D



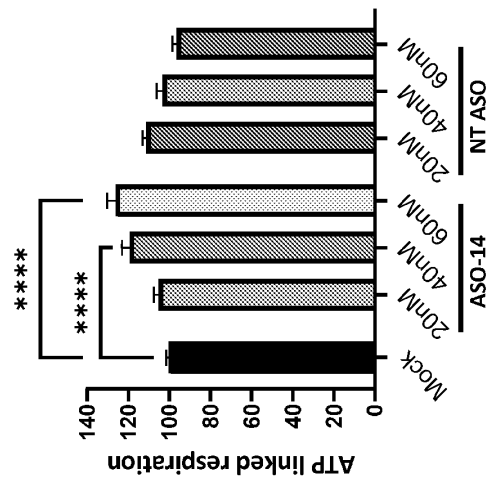


FIG. 28B

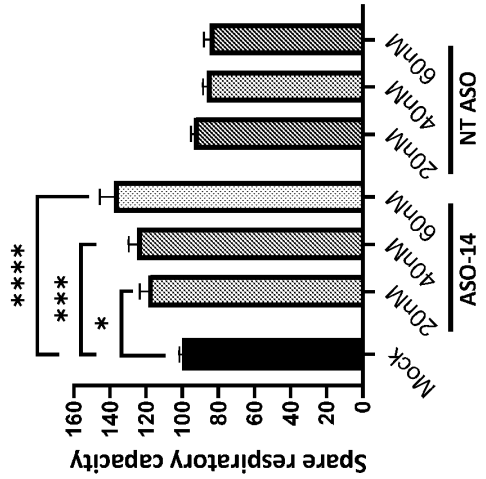


FIG. 28D

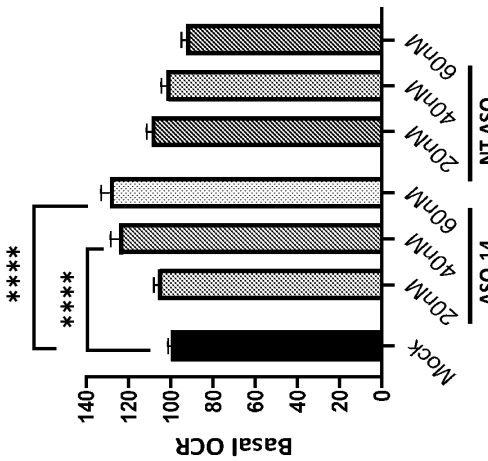


FIG. 28A

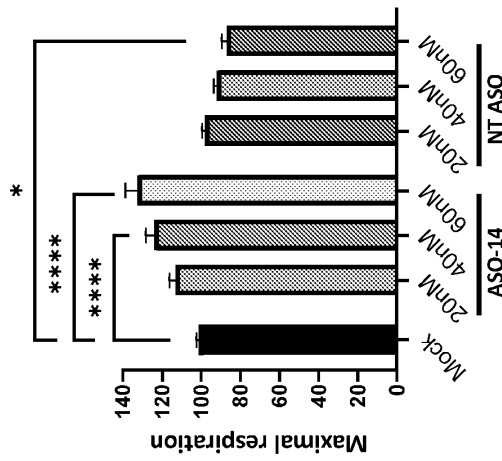


FIG. 28C

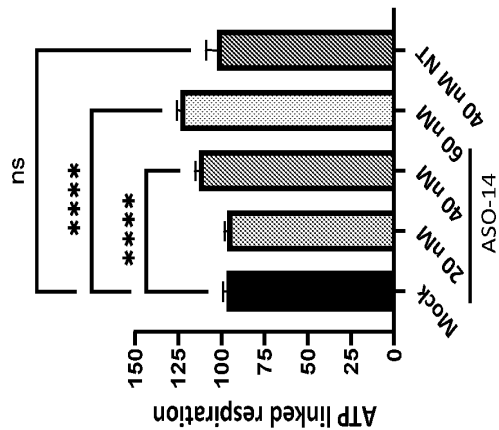


FIG. 29B

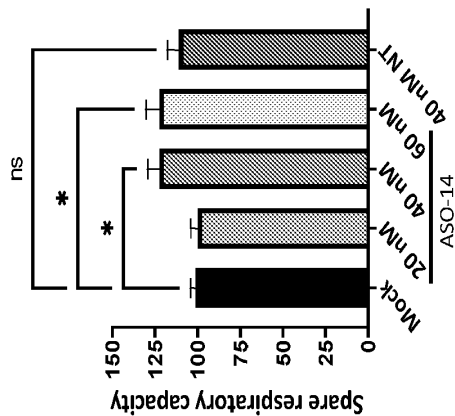


FIG. 29D

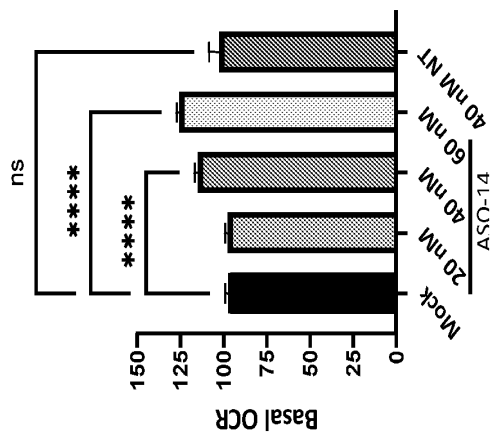


FIG. 29A

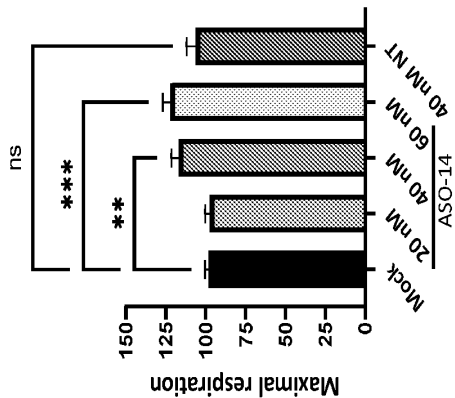


FIG. 29C