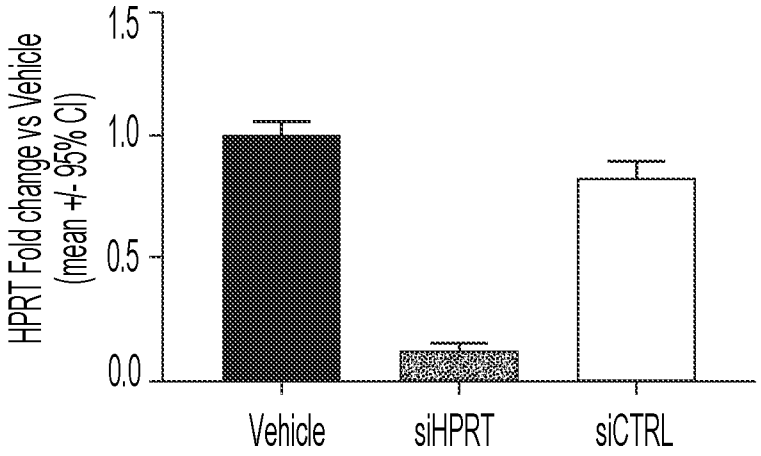
HPRT Fold change vs Vehicle
(mean +/- 95% CI)
1.5
1.0
0.5
0.0
Vehicle
siHPRT
siCTRL

FIG. 1

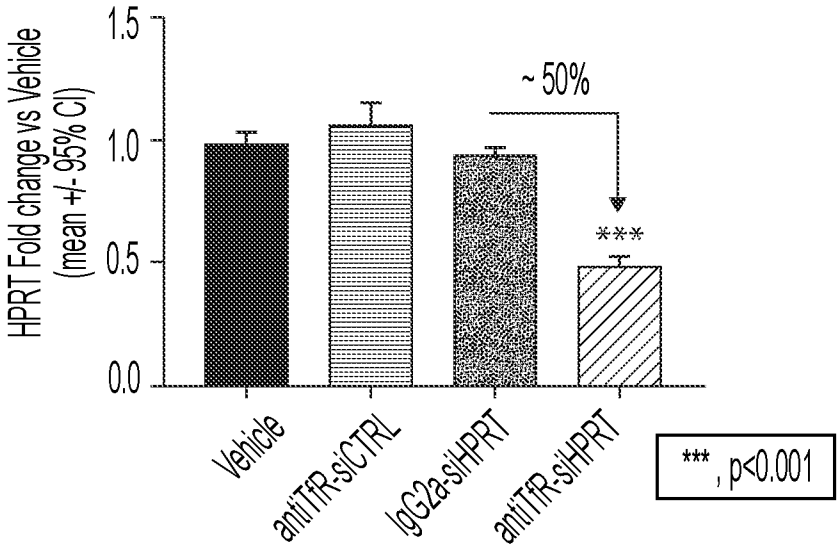
HPRT Fold change vs Vehicle
(mean +/- 95% CI)
1.5
1.0
0.5
0.0
~ 50%
***
Vehicle
antiTfR-siCTRL
IgG2a-siHPRT
antiTfR-siHPRT
***, p<0.001

FIG. 2

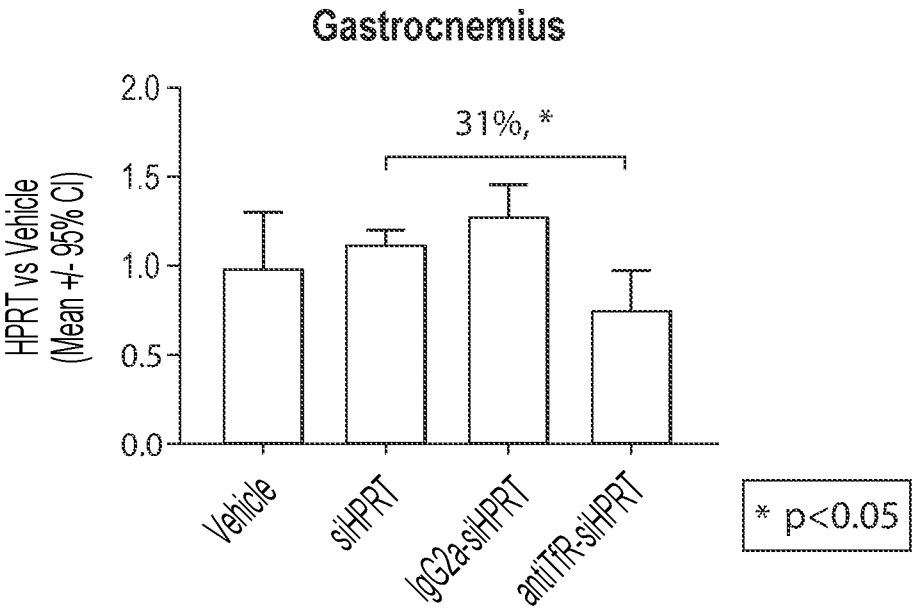
Gastrocnemius
HPRT vs Vehicle
(Mean +/- 95% CI)
2.0
1.5
1.0
0.5
0.0
31%, *
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT
* p<0.05

FIG. 3A

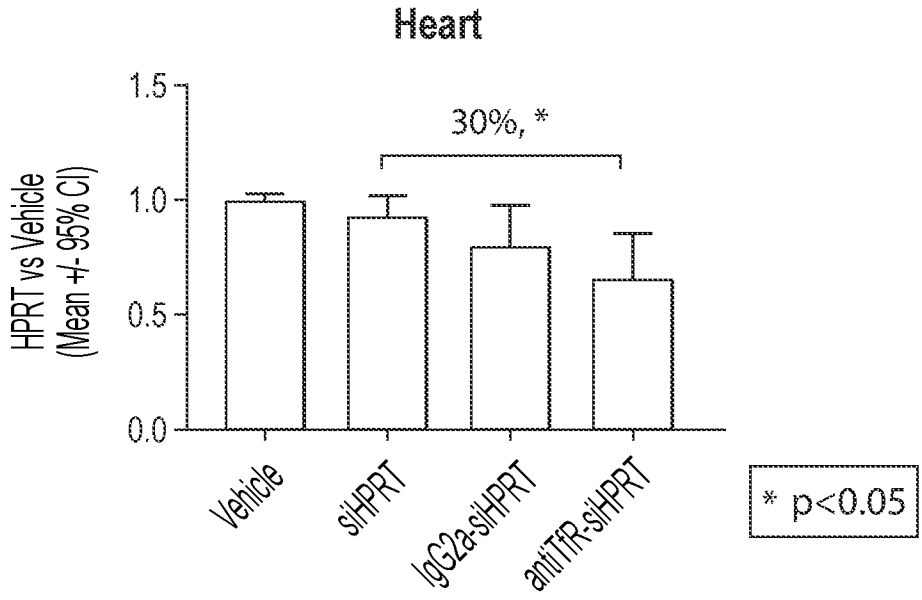
Heart
HPRT vs Vehicle
(Mean +/- 95% CI)
1.5
1.0
0.5
0.0
30%, *
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT
* p<0.05

FIG. 3B

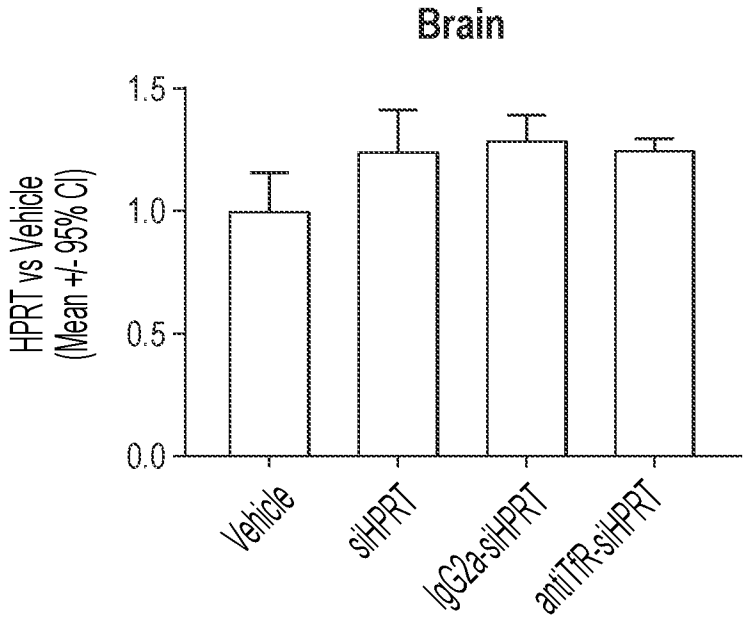
Brain
HPRT vs Vehicle
(Mean +/- 95% CI)
1.5
1.0
0.5
0.0
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT

FIG. 4A

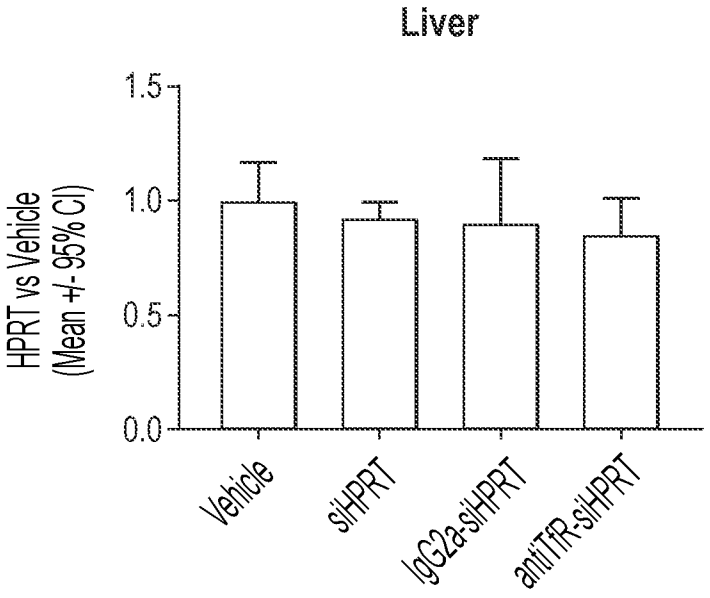
Liver
HPRT vs Vehicle
(Mean +/- 95% CI)
1.5
1.0
0.5
0.0
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT

FIG. 4B

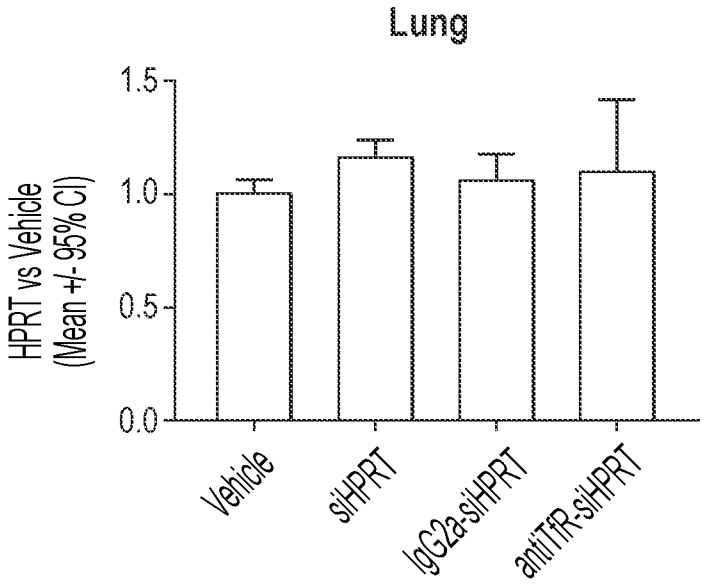
Lung
HPRT vs Vehicle
(Mean +/- 95% CI)
1.5
1.0
0.5
0.0
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT

FIG. 4C

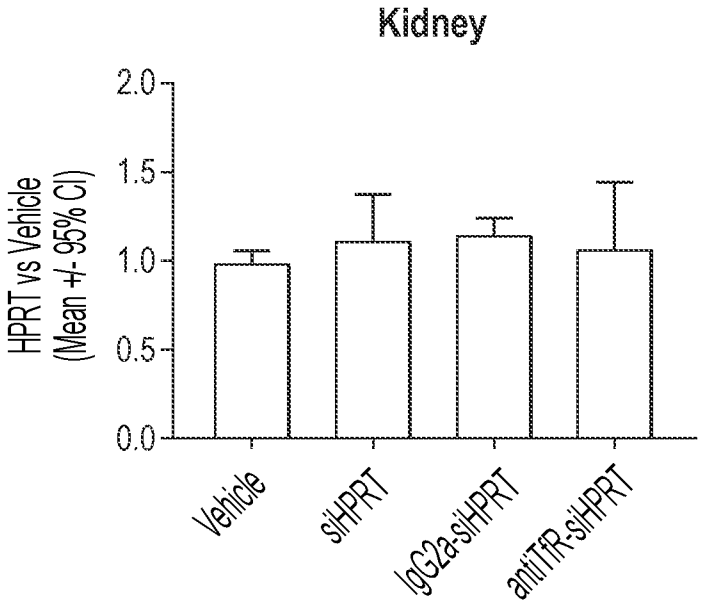
Kidney
HPRT vs Vehicle
(Mean +/- 95% CI)
2.0
1.5
1.0
0.5
0.0
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT

FIG. 4D

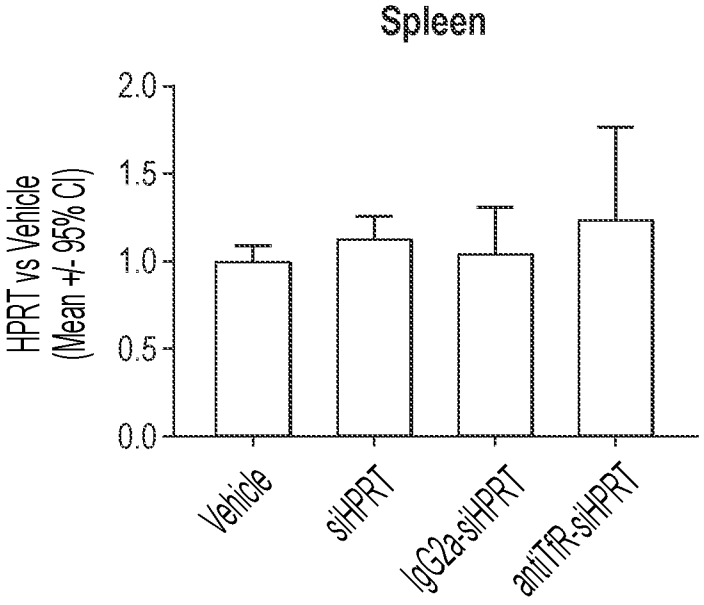
Spleen
HPRT vs Vehicle
(Mean +/- 95% CI)
2.0
1.5
1.0
0.5
0.0
Vehicle
siHPRT
IgG2a-siHPRT
antiTfR-siHPRT

FIG. 4E

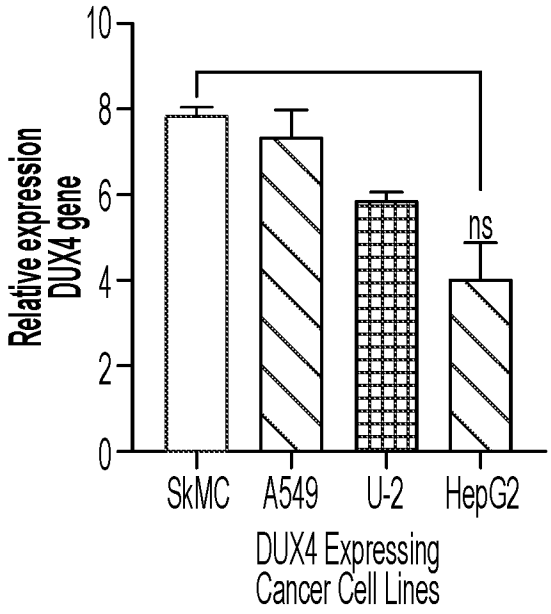
Relative expression
DUX4 gene
10
8
6
4
2
0
ns
SkMC
A549
U-2
HepG2
DUX4 Expressing
Cancer Cell Lines

FIG. 5

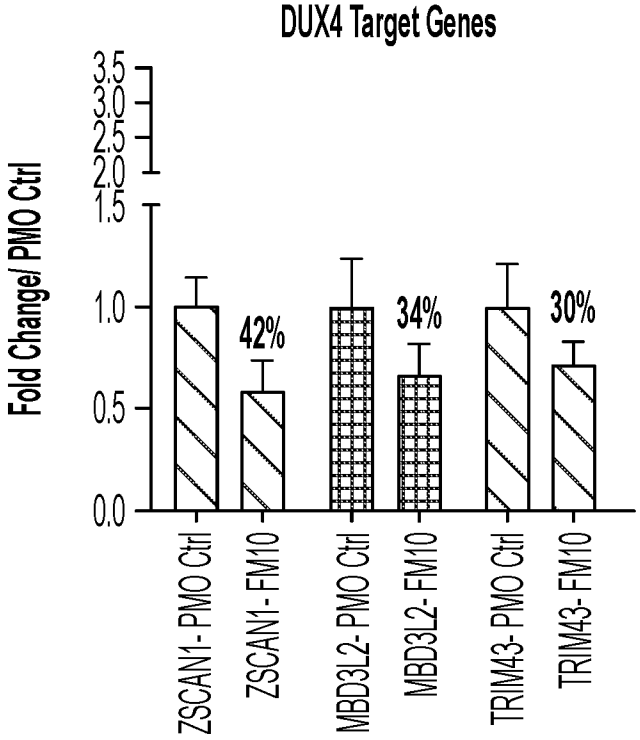
DUX4 Target Genes
Fold Change/ PMO Ctrl
3.5
3.0
2.5
2.0
1.5
1.0
0.5
0.0
42%
34%
30%
ZSCAN1- PMO Ctrl
ZSCAN1- FM10
MBD3L2- PMO Ctrl
MBD3L2- FM10
TRIM43- PMO Ctrl
TRIM43- FM10

FIG. 6

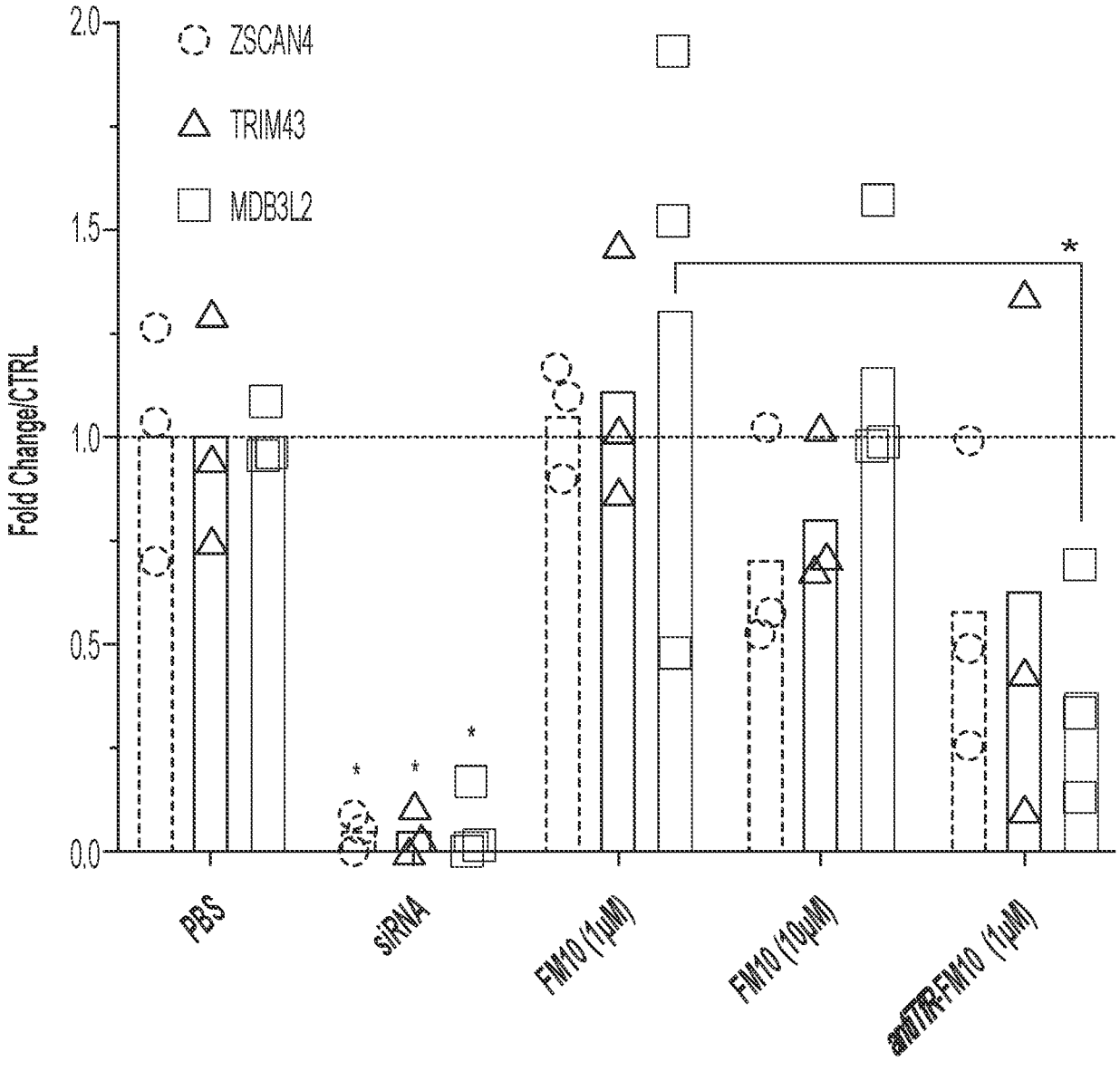
2.0
1.5
1.0
0.5
0.0
Fold Change/CTRL
ZSCAN4
TRIM43
MDB3L2
*
*
*
*
PBS
siRNA
FM10 (1μM)
FM10 (10μM)
antiTRFM10 (1μM)

FIG. 7

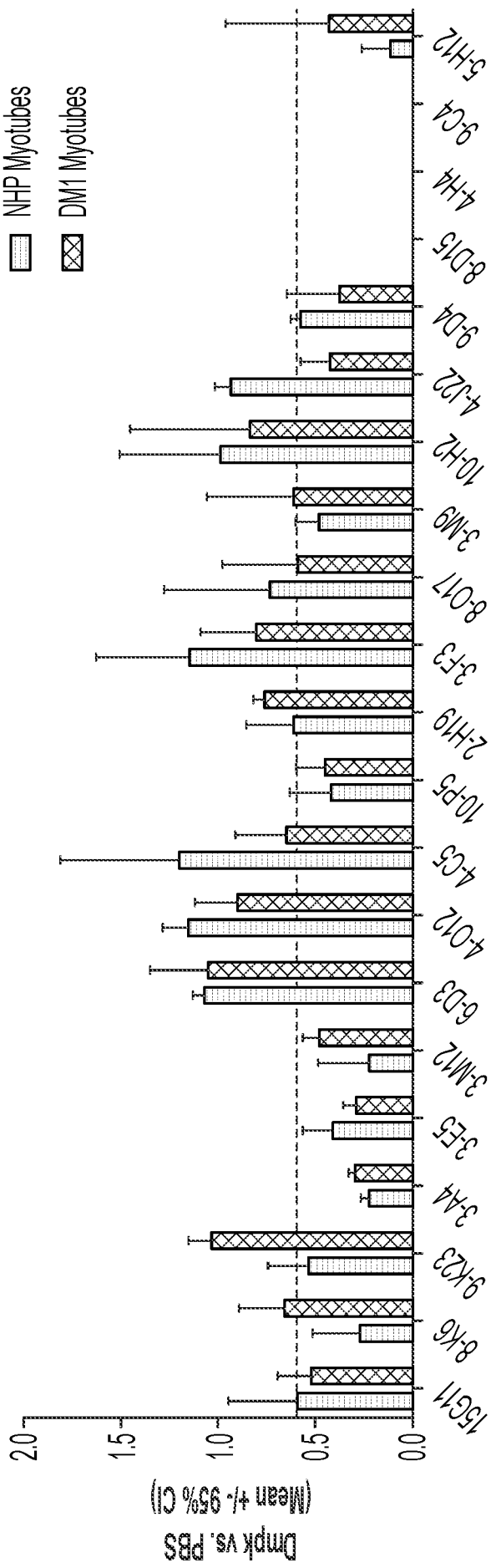
NHP Myotubes
DM1 Myotubes
Dmpk vs. PBS
(Mean +/- 95% CI)
2.0
1.5
1.0
0.5
0.0
15C11
8-K6
9-K23
3-A4
3-E5
3-M12
6-D3
4-O12
4-C5
10-P5
2-H19
3-F3
8-O17
3-M9
10-H2
4-J22
9-D4
8-D15
4-H4
9-C4
5-H12

FIG. 8

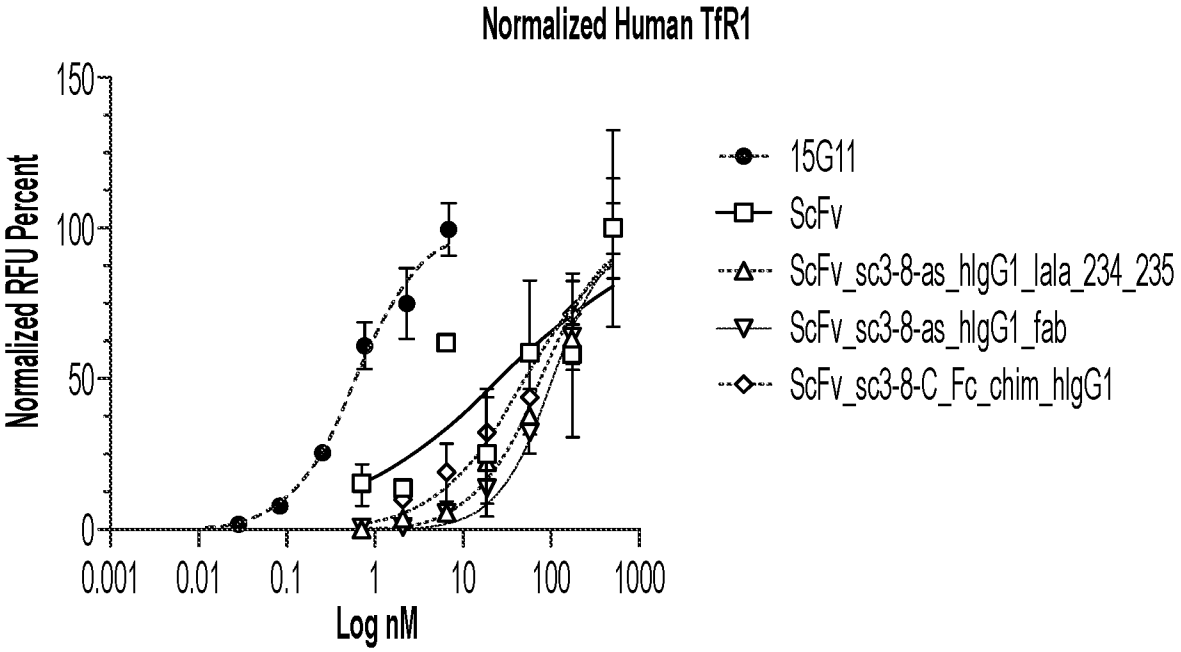
Normalized Human TfR1
Normalized RFU Percent
150
100
50
0
0.001
0.01
0.1
1
10
100
1000
Log nM
15G11
ScFv
ScFv_sc3-8-as_hIgG1_lala_234_235
ScFv_sc3-8-as_hIgG1_fab
ScFv_sc3-8-C_Fc_chim_hIgG1

FIG. 9A

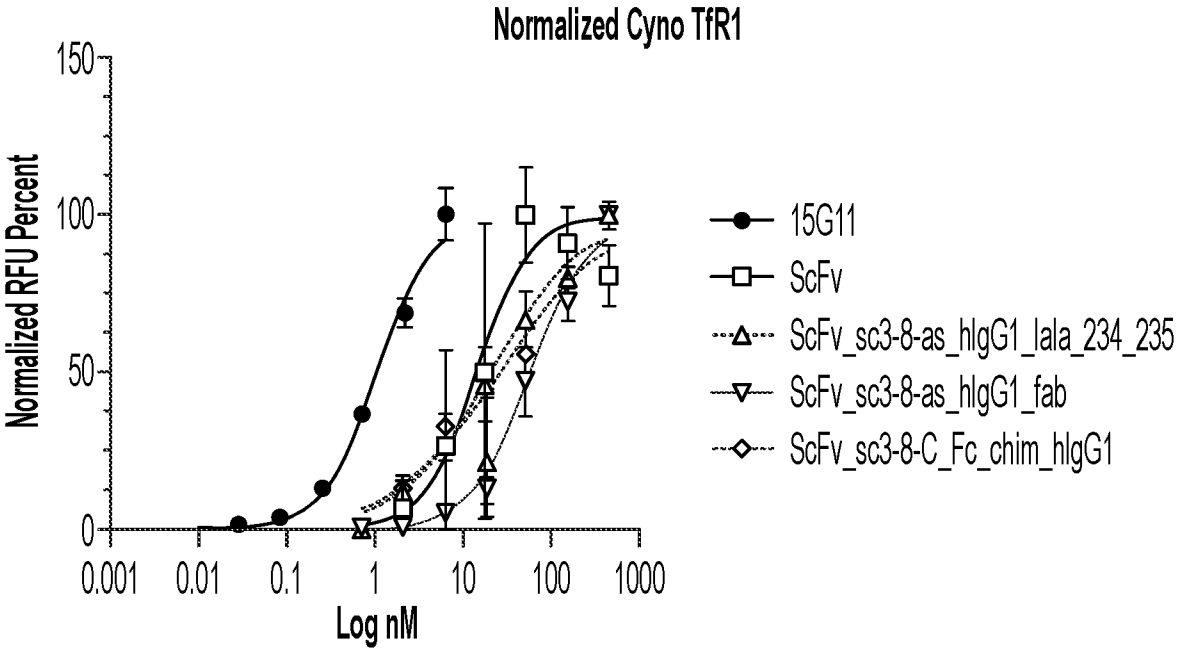
Normalized Cyno TfR1
Normalized RFU Percent
150
100
50
0
0.001
0.01
0.1
1
10
100
1000
Log nM
15G11
ScFv
ScFv_sc3-8-as_hIgG1_lala_234_235
ScFv_sc3-8-as_hIgG1_fab
ScFv_sc3-8-C_Fc_chim_hIgG1

FIG. 9B

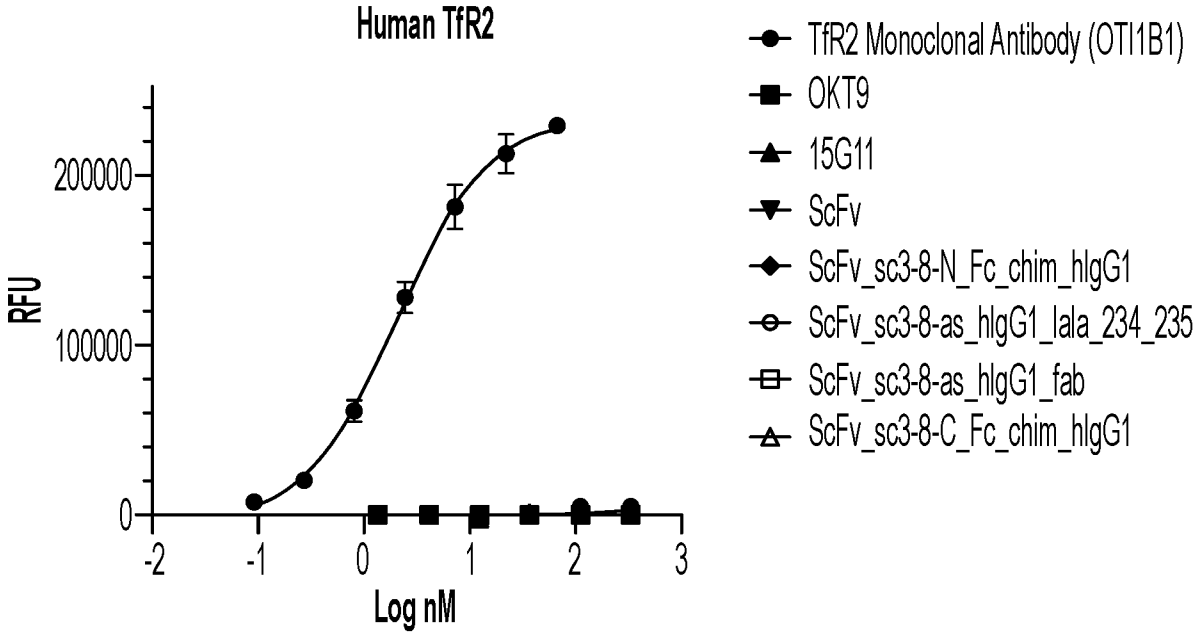
Human TfR2
RFU
200000
100000
0
-2
-1
0
1
2
3
Log nM
TfR2 Monoclonal Antibody (OTI1B1)
OKT9
15G11
ScFv
ScFv_sc3-8-N_Fc_chim_hIgG1
ScFv_sc3-8-as_hIgG1_lala_234_235
ScFv_sc3-8-as_hIgG1_fab
ScFv_sc3-8-C_Fc_chim_hIgG1

FIG. 10

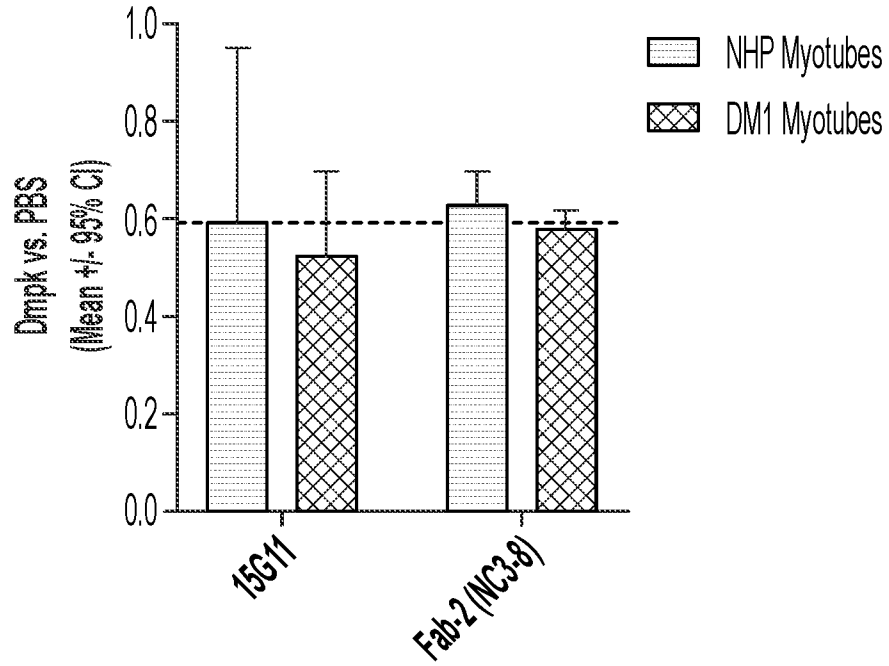
Dmpk vs. PBS
(Mean +/- 95% CI)
1.0
0.8
0.6
0.4
0.2
0.0
15G11
Fab-2(NC3-8)
NHP Myotubes
DM1 Myotubes

FIG. 11

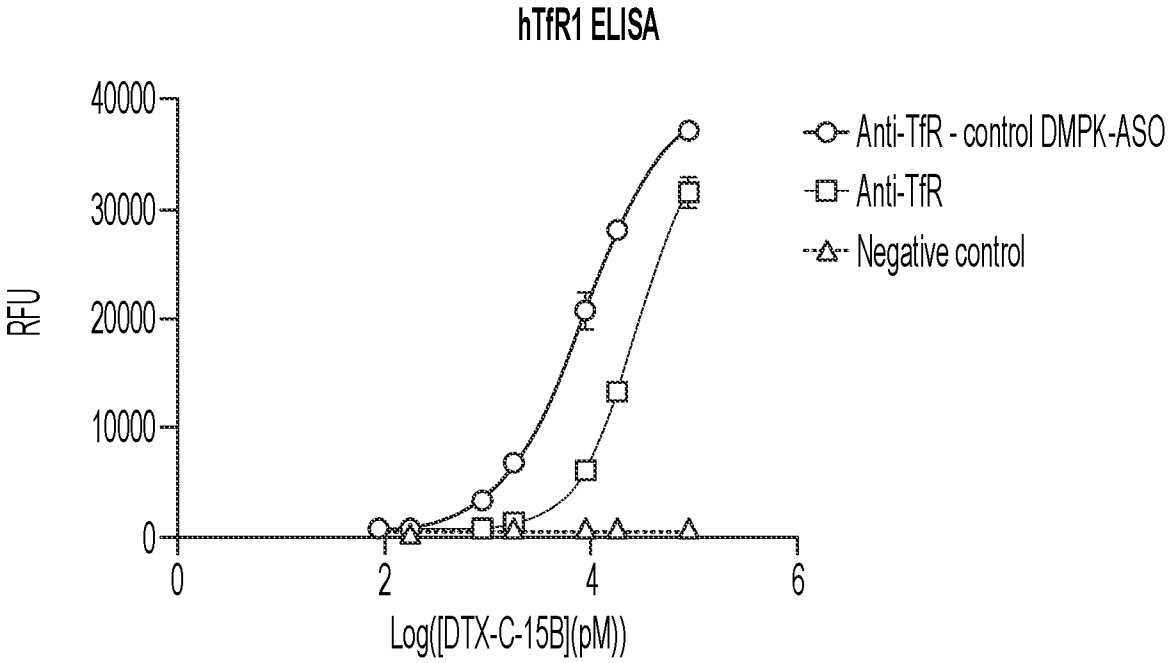

FIG. 12A

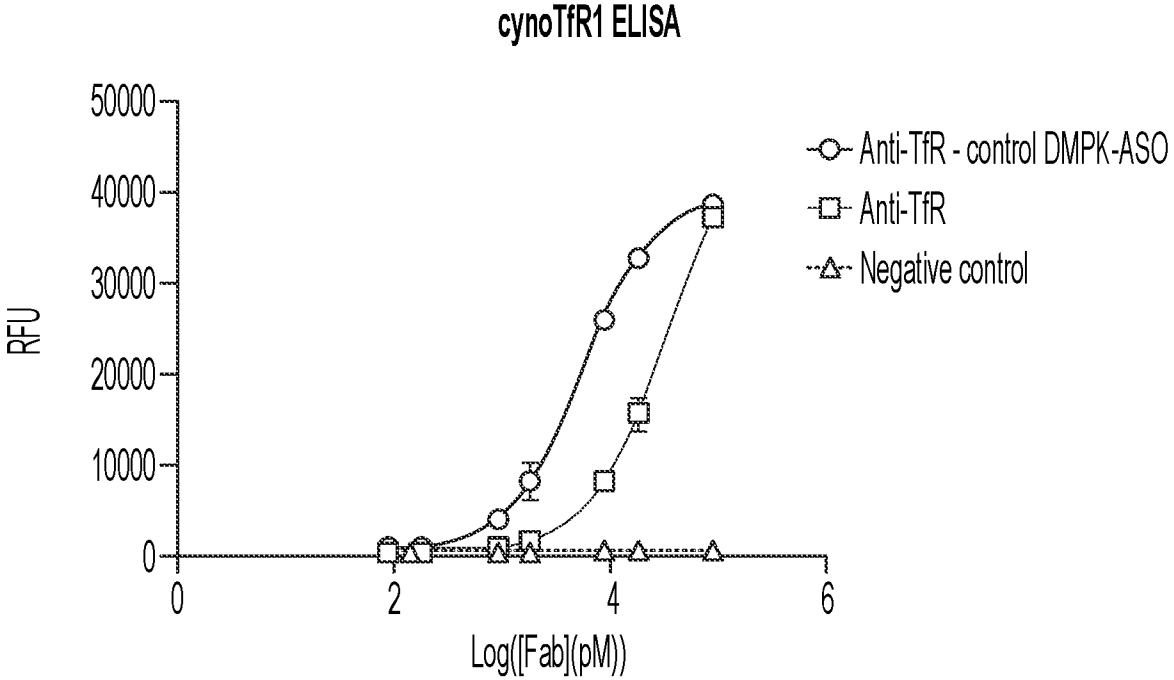

FIG. 12B

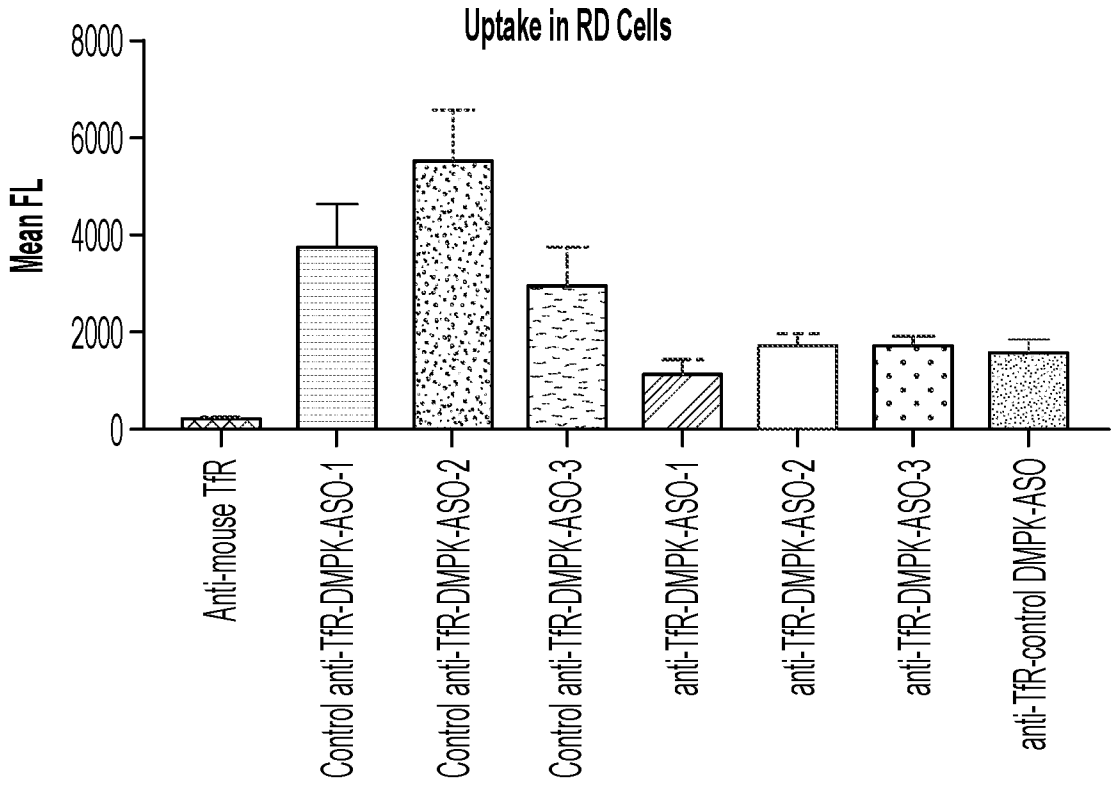
Uptake in RD Cells
Mean FL
8000
6000
4000
2000
0
Anti-mouse TfR
Control anti-TfR-DMPK-ASO-1
Control anti-TfR-DMPK-ASO-2
Control anti-TfR-DMPK-ASO-3
anti-TfR-DMPK-ASO-1
anti-TfR-DMPK-ASO-2
anti-TfR-DMPK-ASO-3
anti-TfR-control DMPK-ASO

FIG. 13

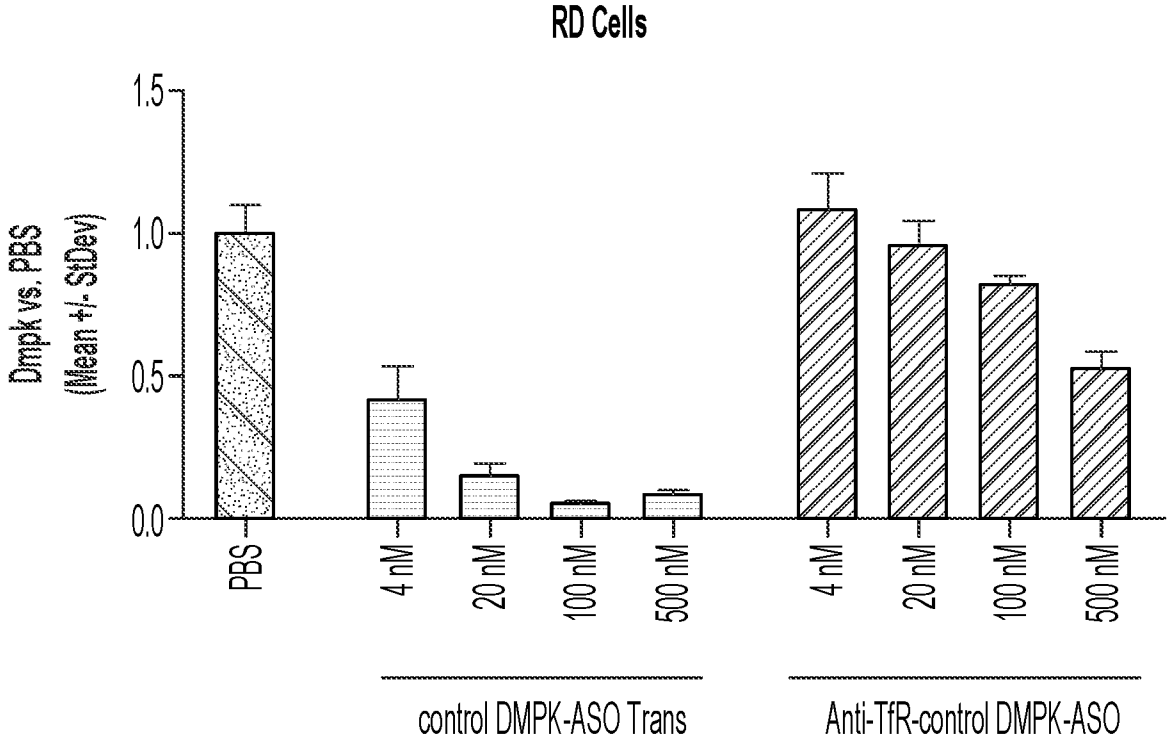
RD Cells
Dmpk vs. PBS
(Mean +/- StDev)
1.5
1.0
0.5
0.0
PBS
4 nM
20 nM
100 nM
500 nM
4 nM
20 nM
100 nM
500 nM
control DMPK-ASO Trans
Anti-TfR-control DMPK-ASO

FIG. 14

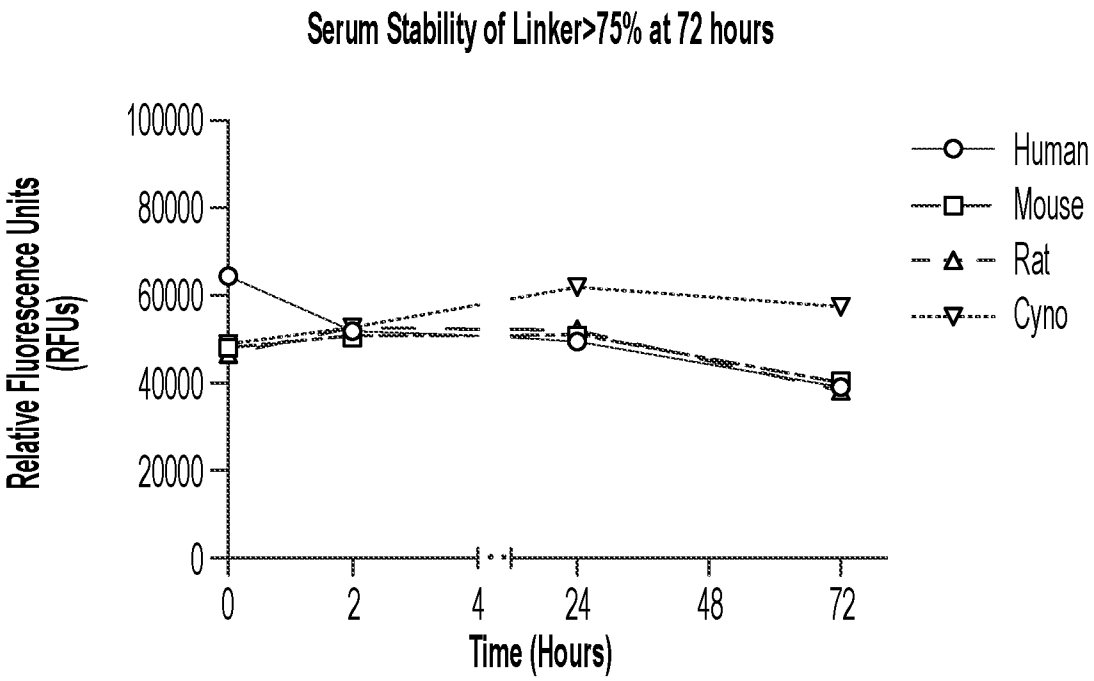
Serum Stability of Linker>75% at 72 hours
Relative Fluorescence Units (RFUs)
100000
80000
60000
40000
20000
0
0
2
4
24
48
72
Time (Hours)
Human
Mouse
Rat
Cyno

FIG. 15

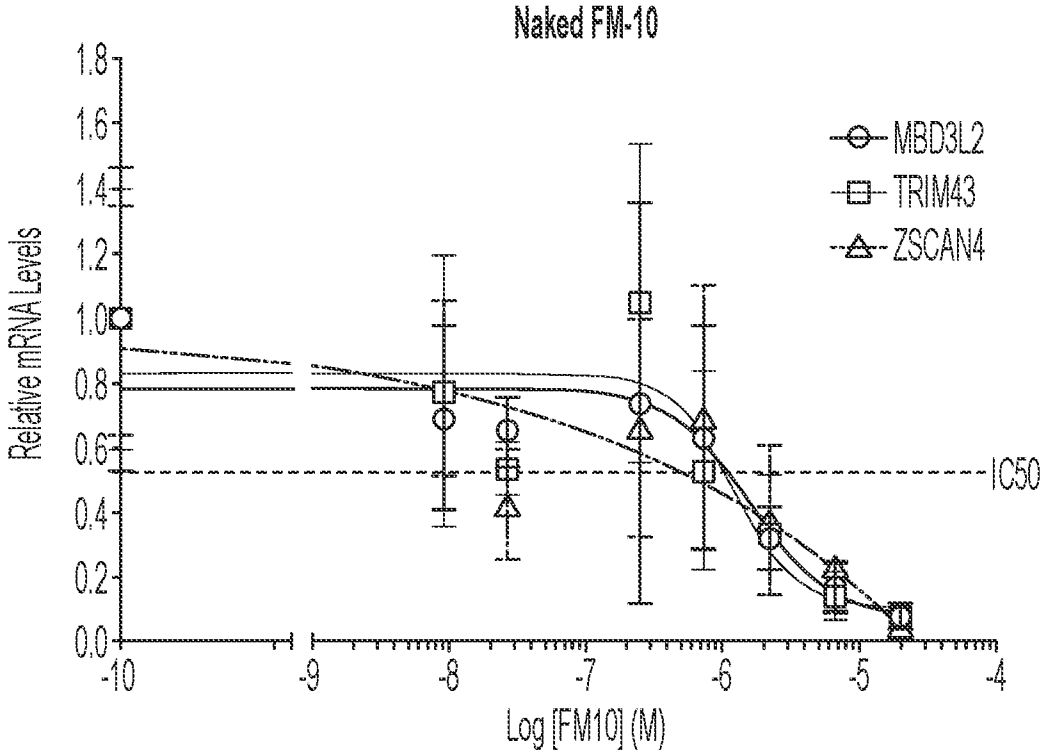

FIG. 16A

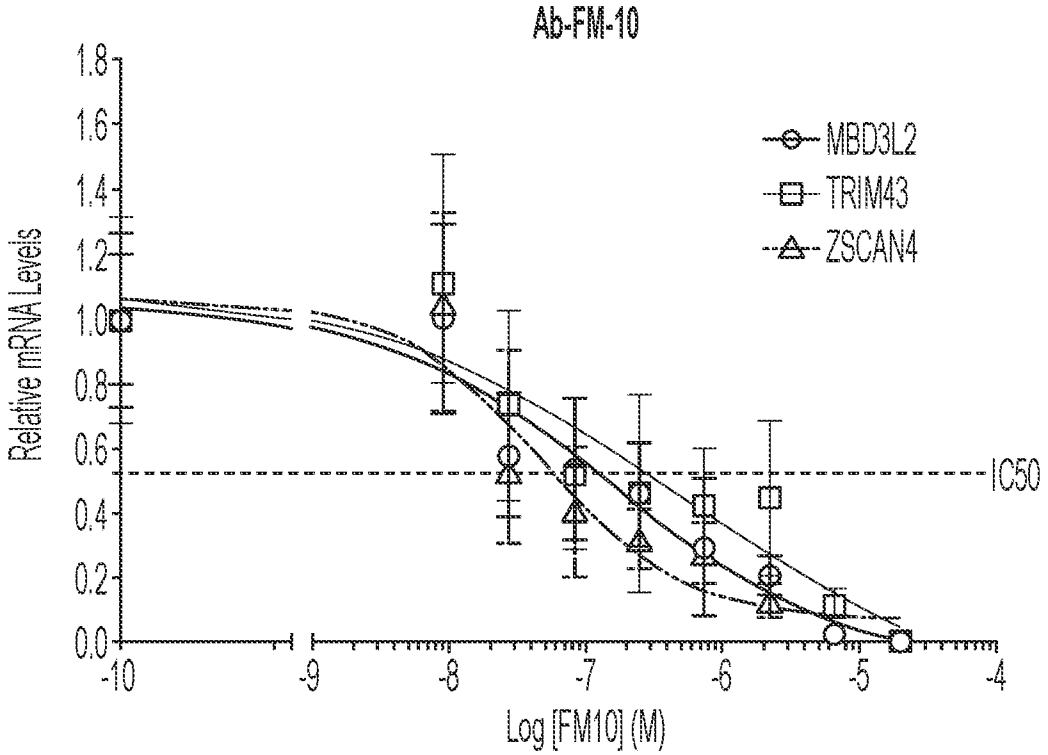

FIG. 16B

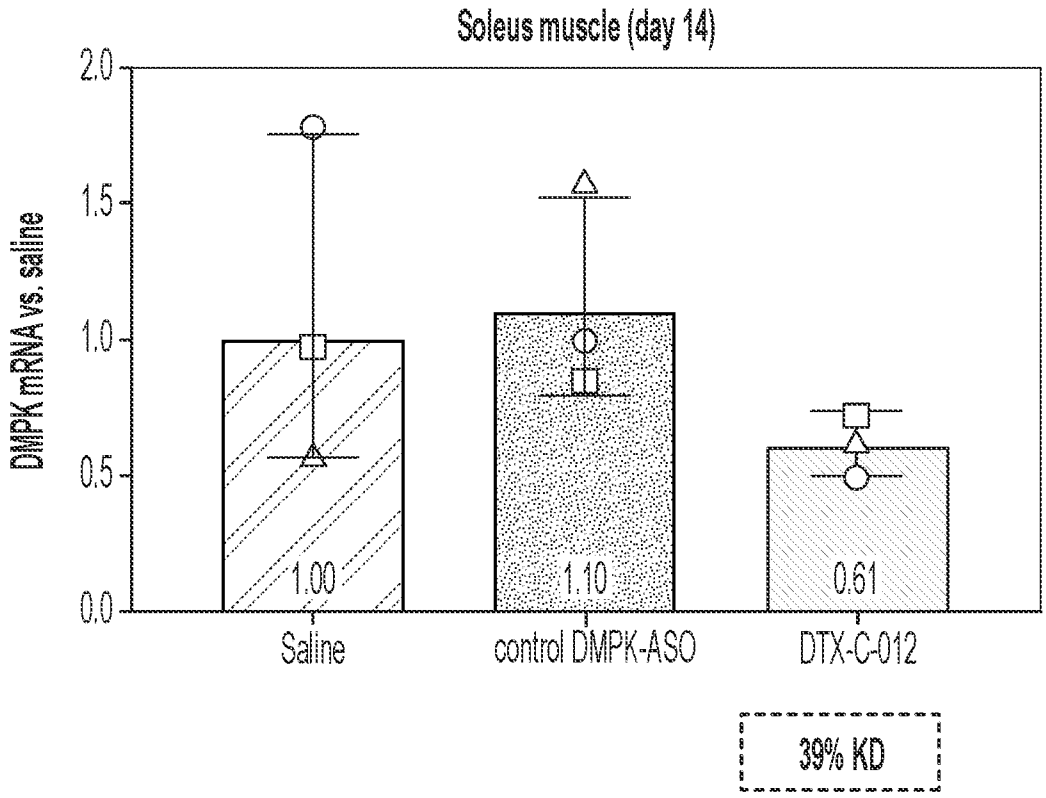
Soleus muscle (day 14)
DMPK mRNA vs. saline
2.0
1.5
1.0
0.5
0.0
1.00
1.10
0.61
Saline
control DMPK-ASO
DTX-C-012
39% KD

FIG. 17A

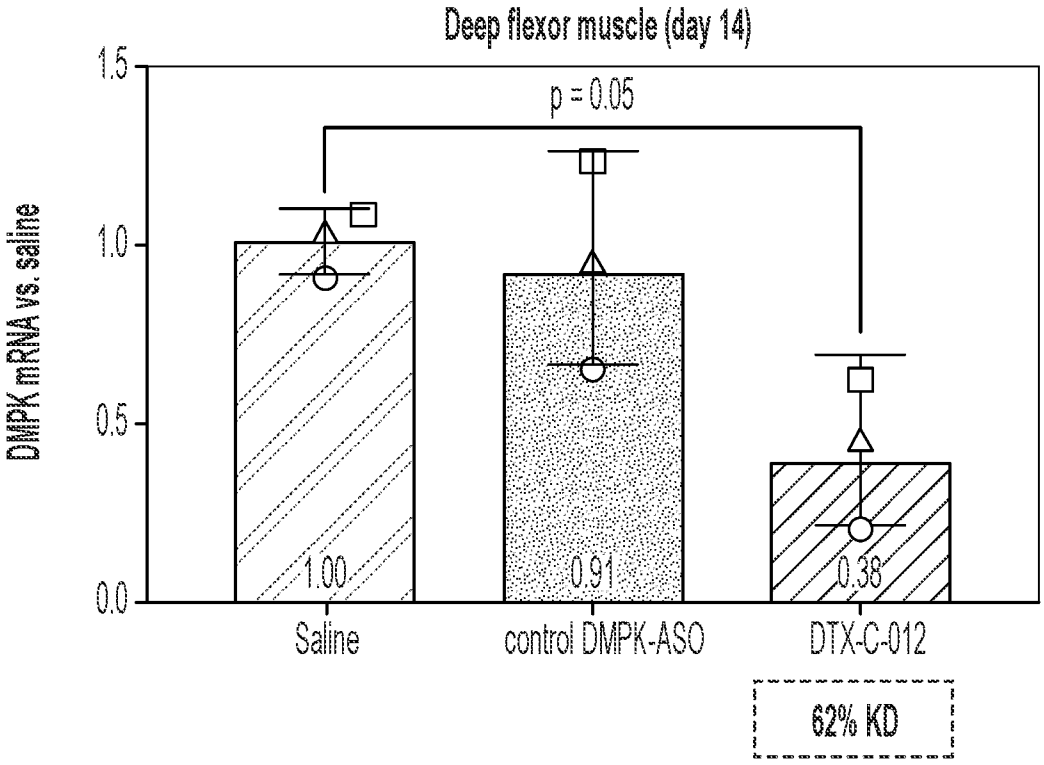

FIG. 17B

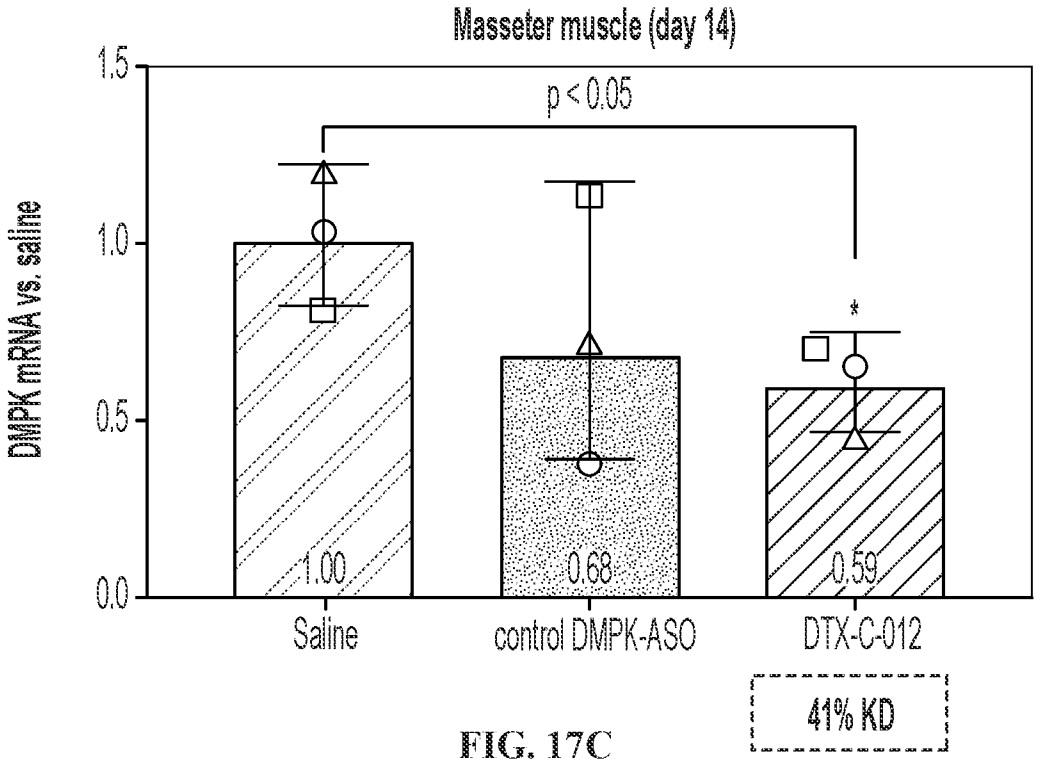

FIG. 17C

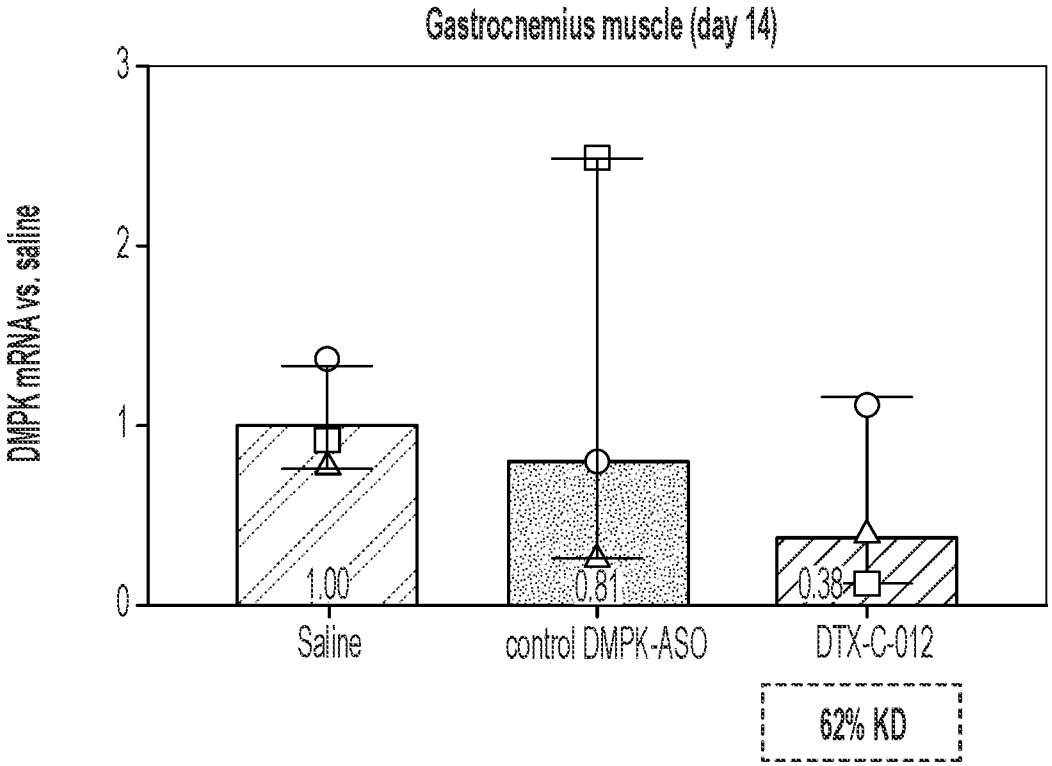

FIG. 17D

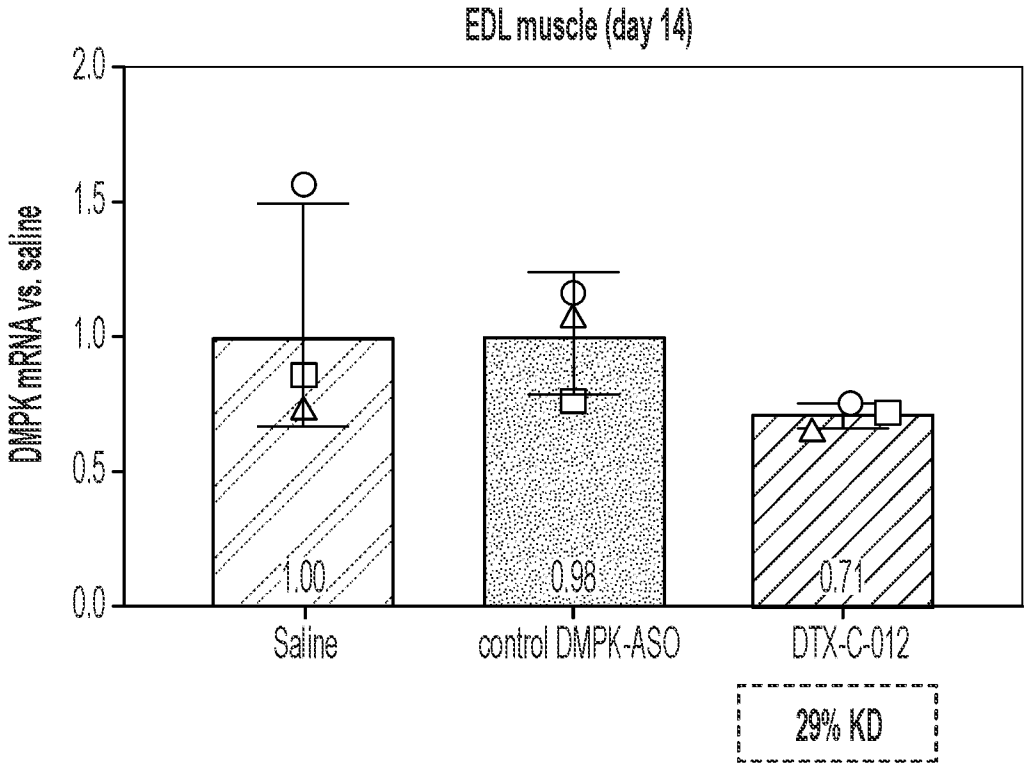

FIG. 17E

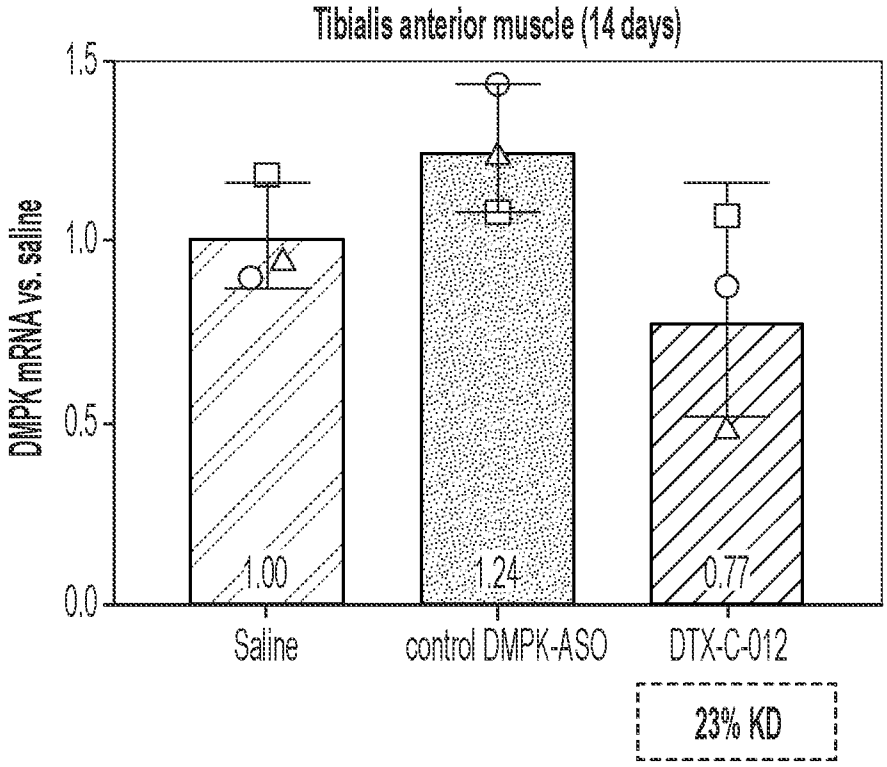

FIG. 17F

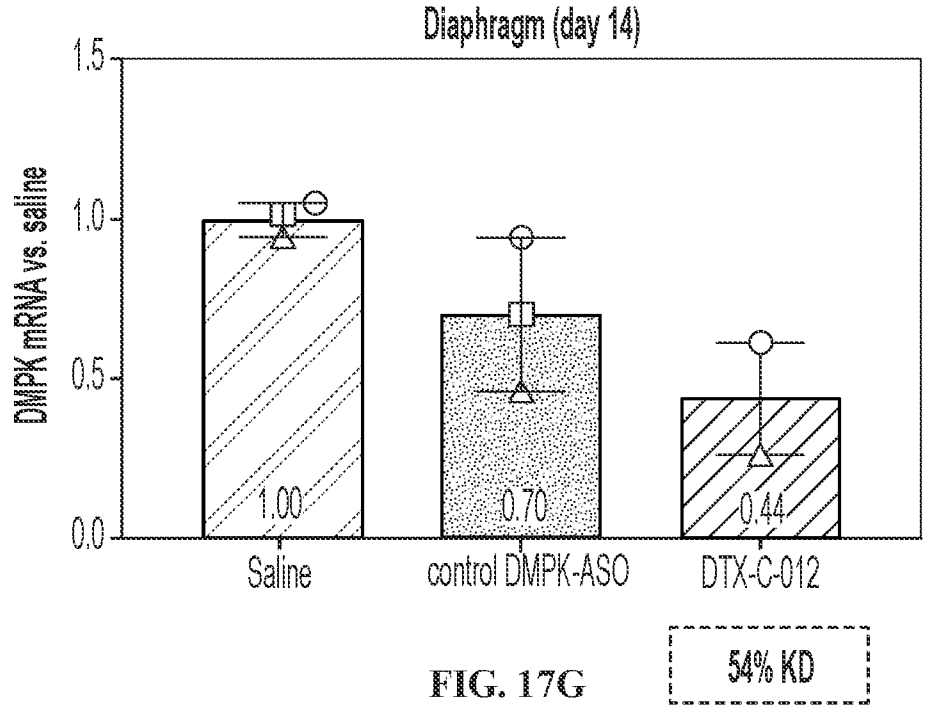

FIG. 17G

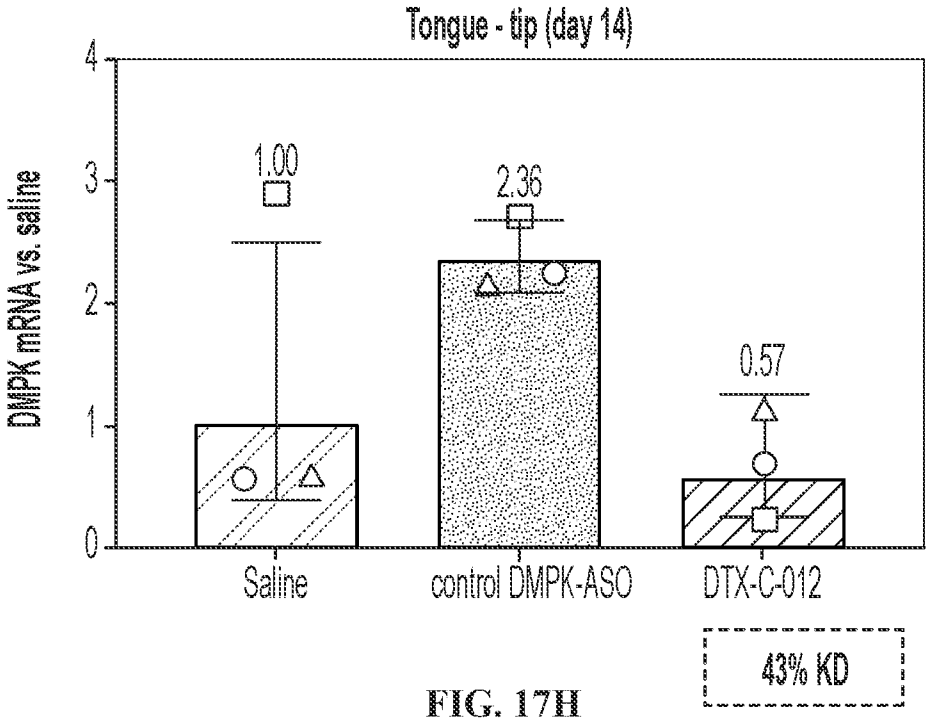

FIG. 17H

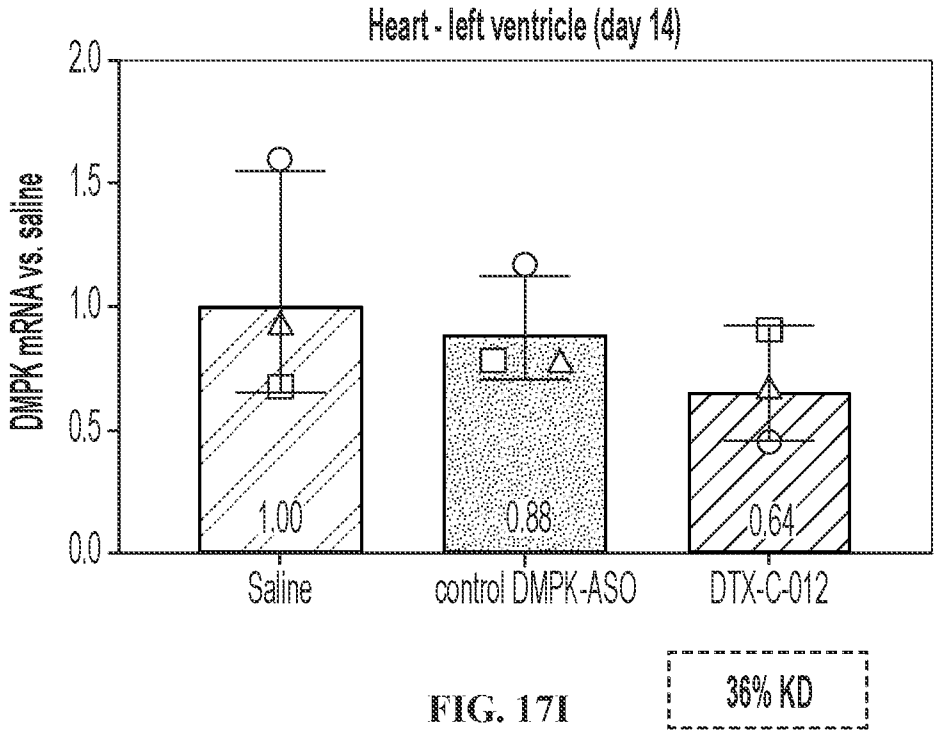

FIG. 17I

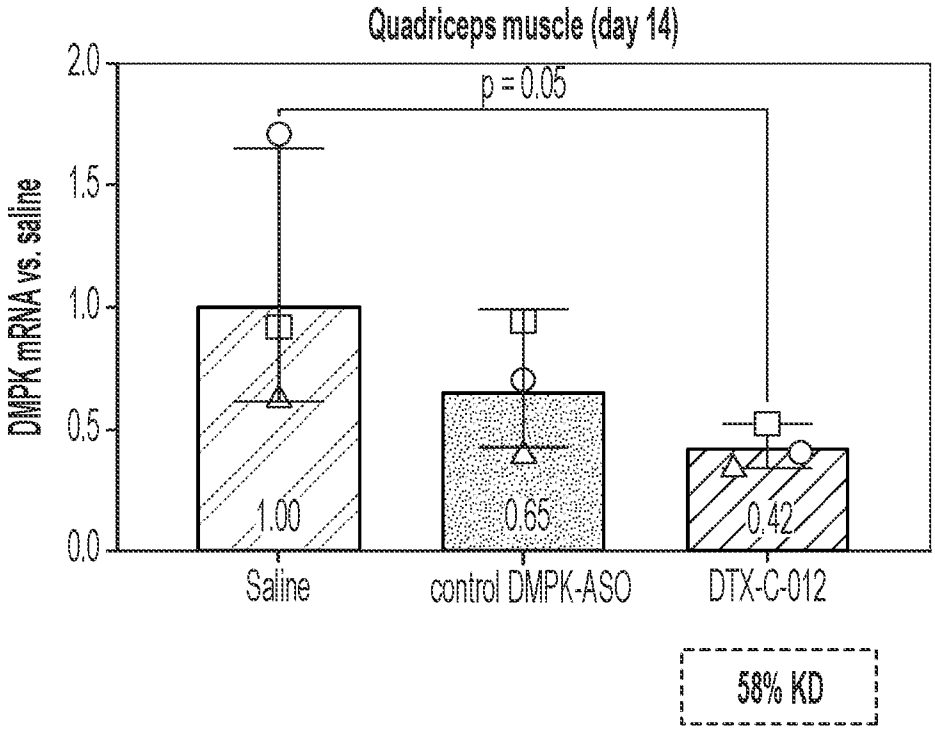

FIG. 17J

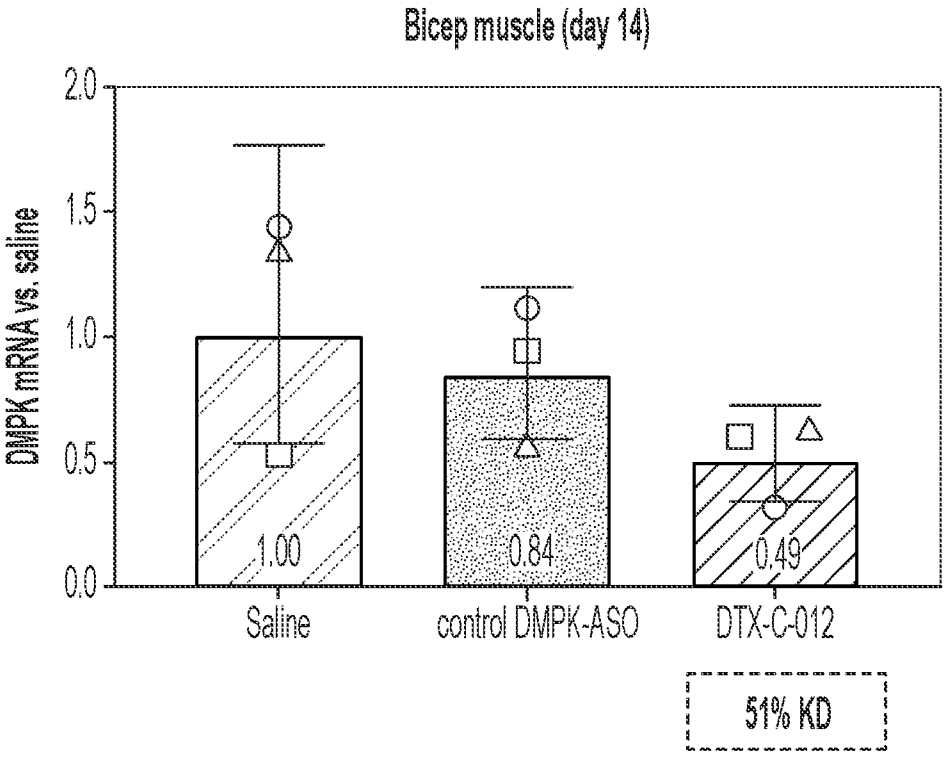

FIG. 17K

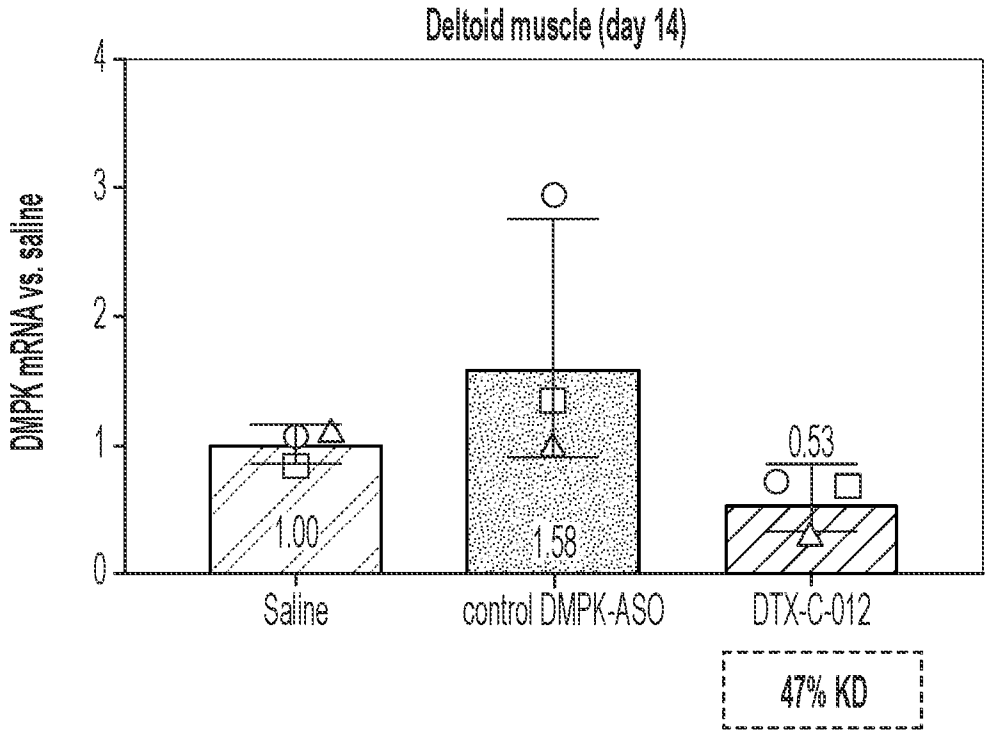

FIG. 17L

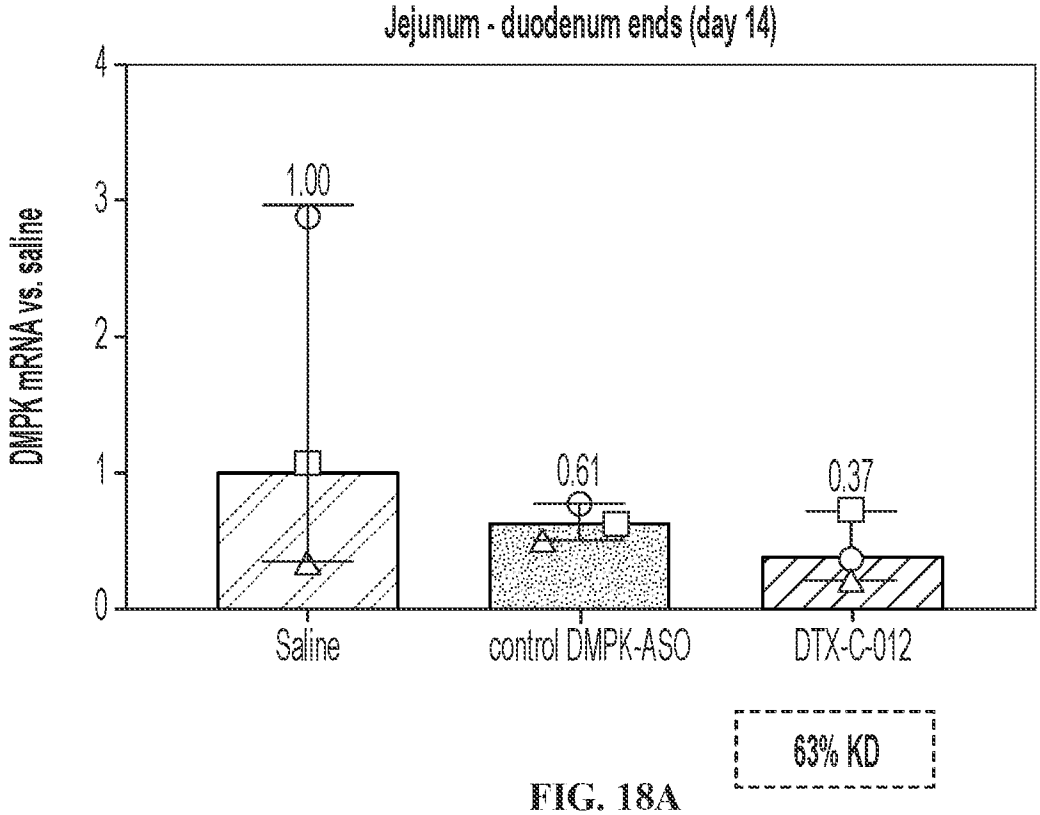

FIG. 18A

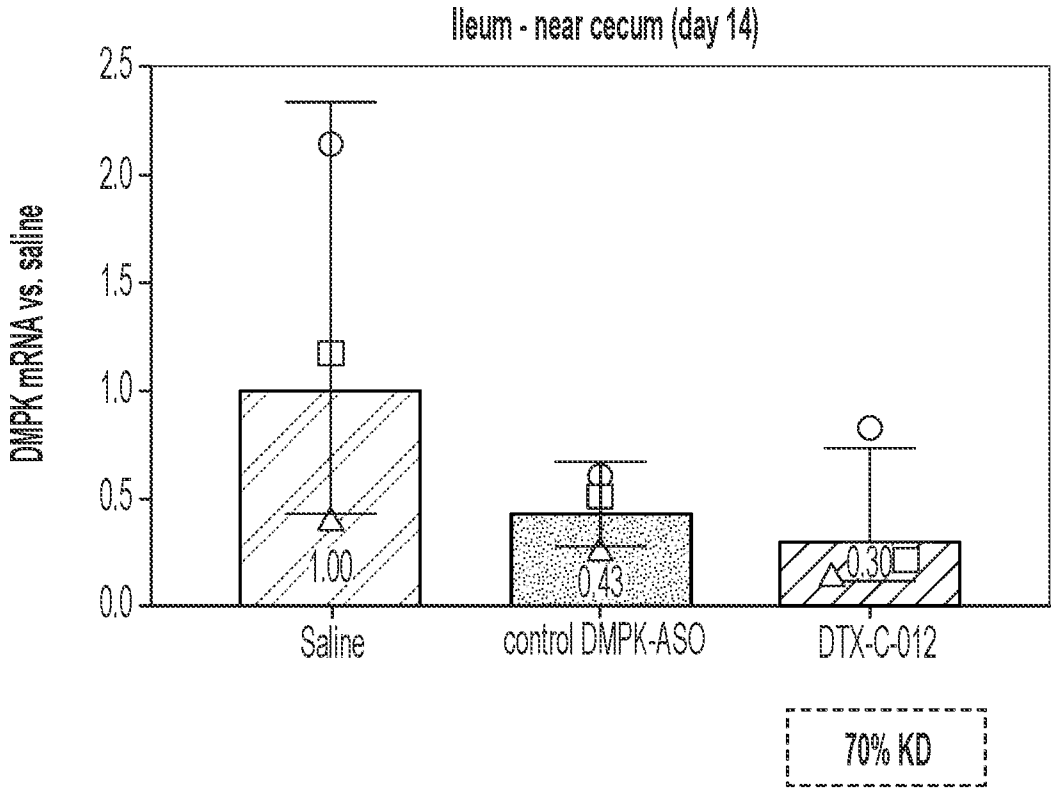

FIG. 18B

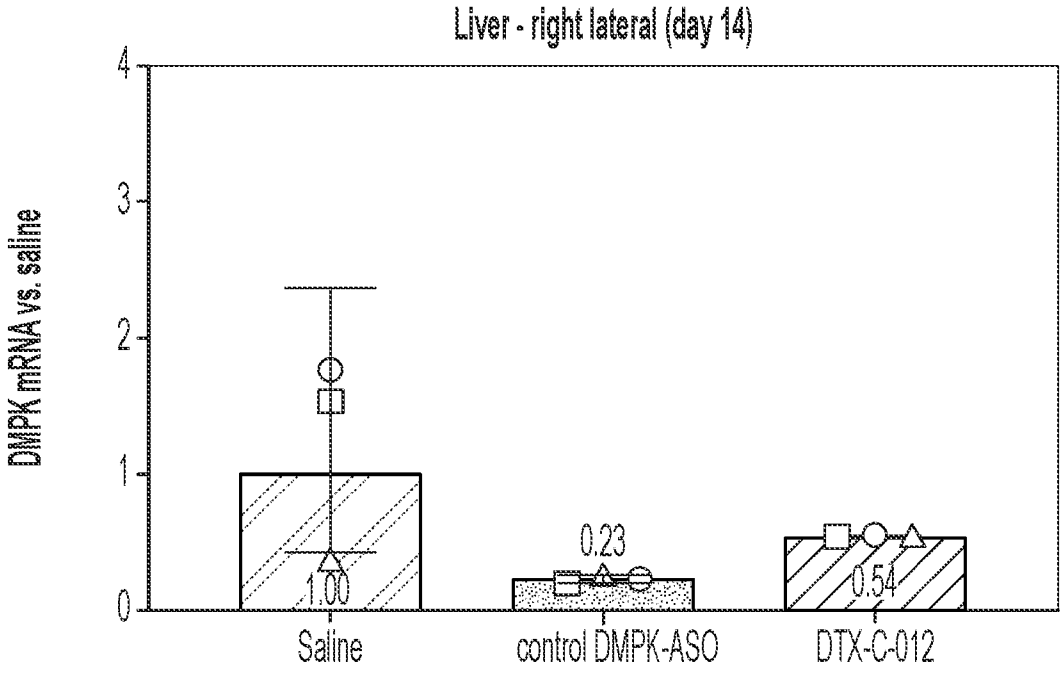

FIG. 19A

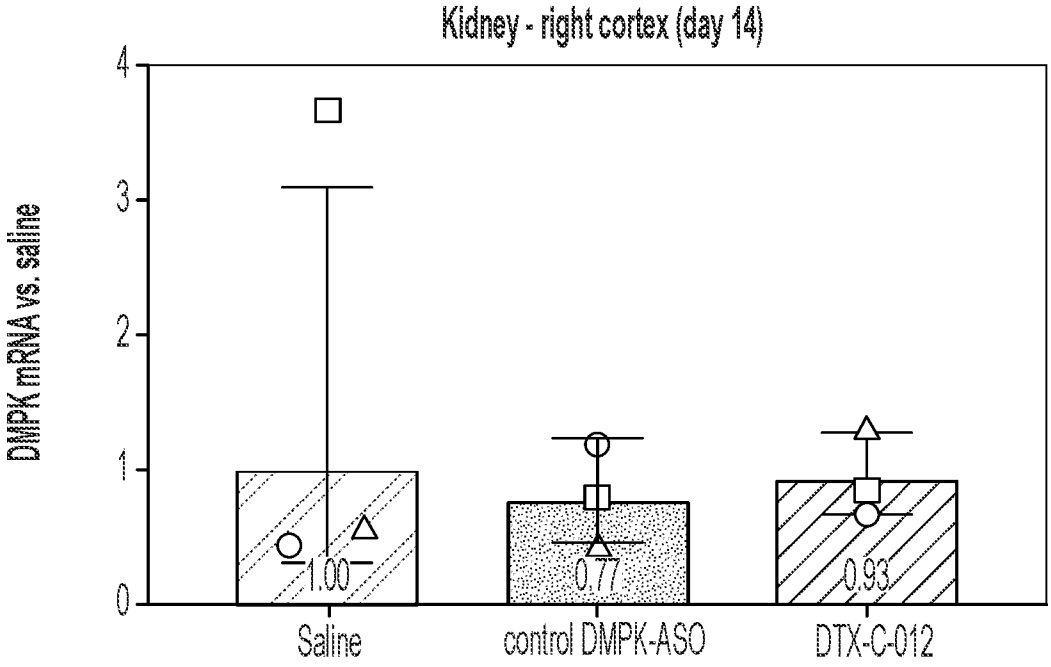

FIG. 19B

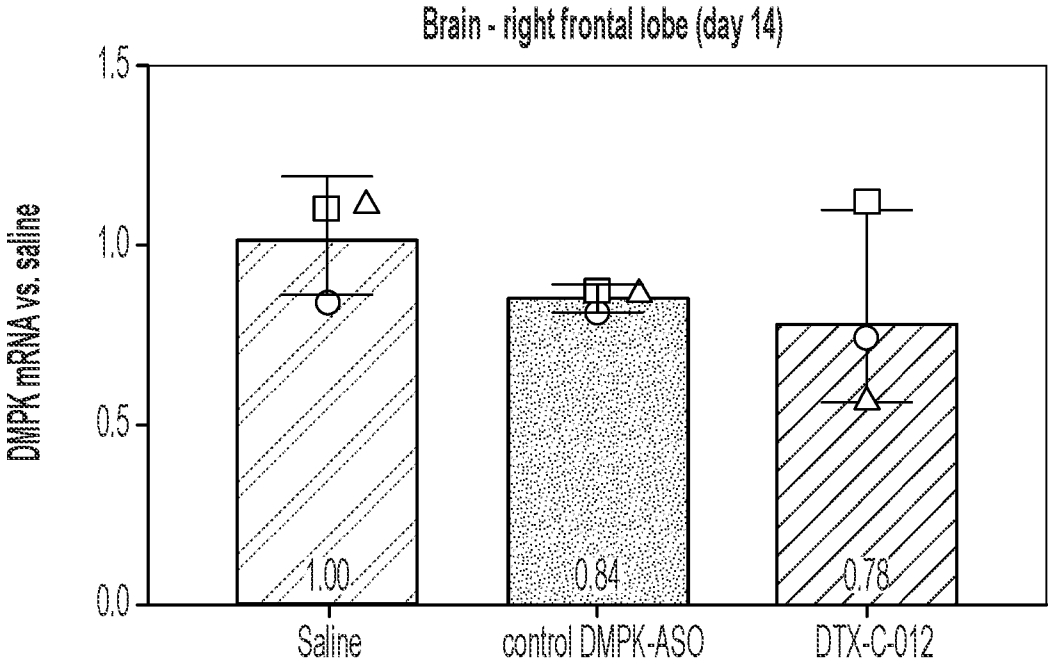

FIG. 19C

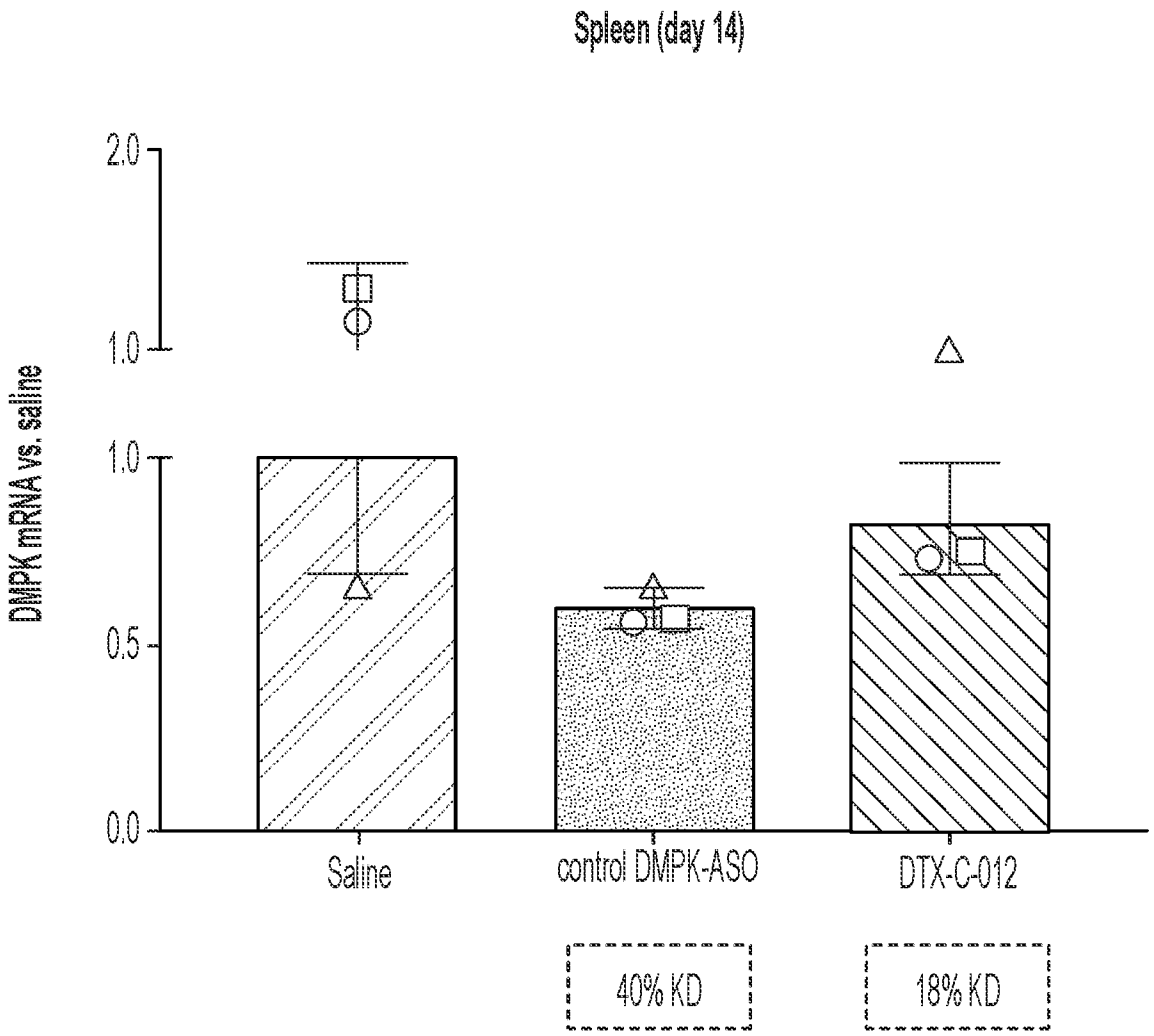
Spleen (day 14)
DMPK mRNA vs. saline
2.0
1.0
1.0
0.5
0.0
Saline
control DMPK-ASO
DTX-C-012
40% KD
18% KD

FIG. 19D

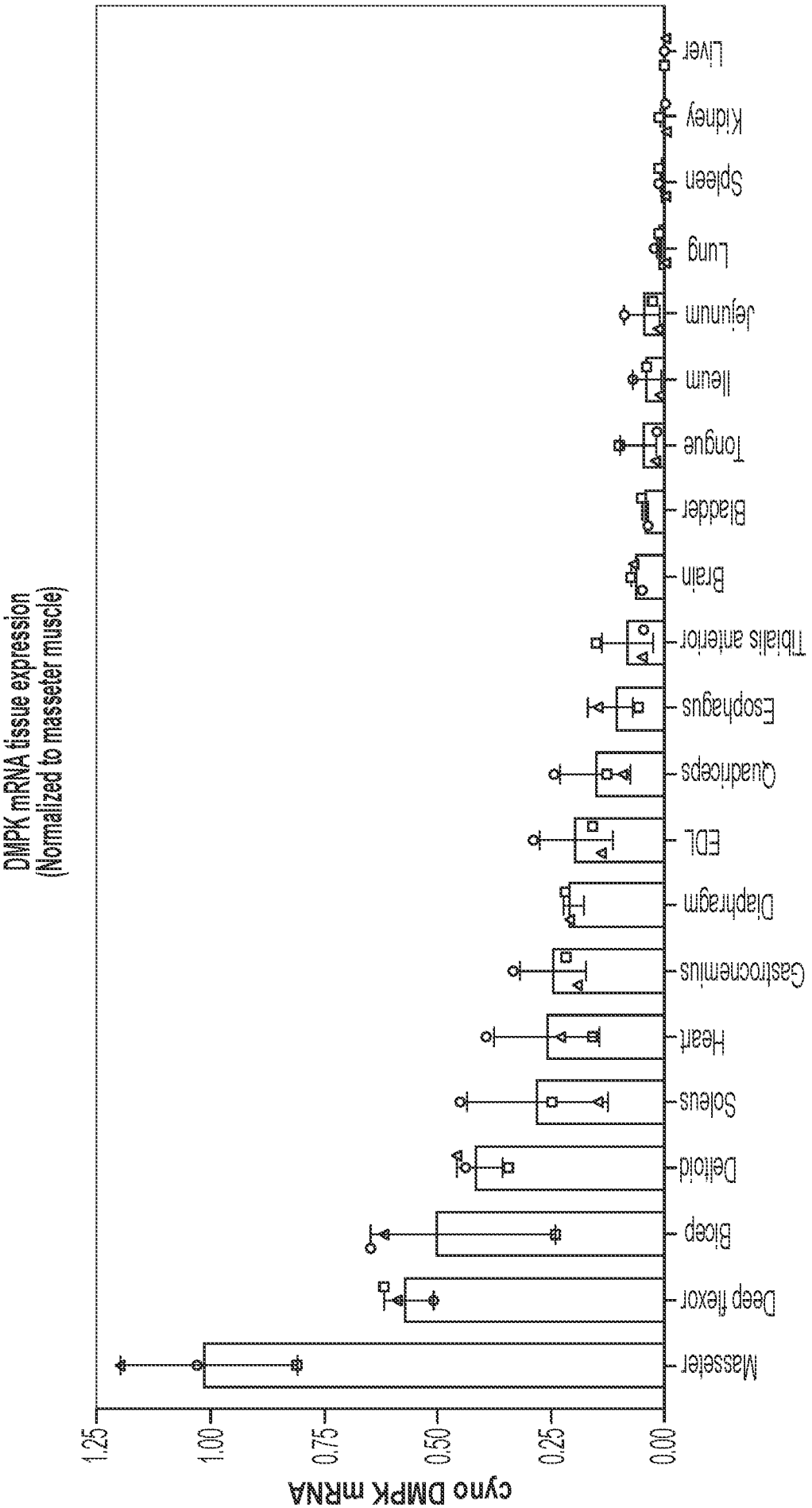
DMPK mRNA tissue expression
(Normalized to masseter muscle)
cyno DMPK mRNA
1.25
1.00
0.75
0.50
0.25
0.00
Masseter
Deep flexor
Bicep
Deltoid
Soleus
Heart
Gastrocnemius
Diaphragm
EDL
Quadriceps
Esophagus
Tibialis anterior
Brain
Bladder
Tongue
Ileum
Jejunum
Lung
Spleen
Kidney
Liver

FIG. 20

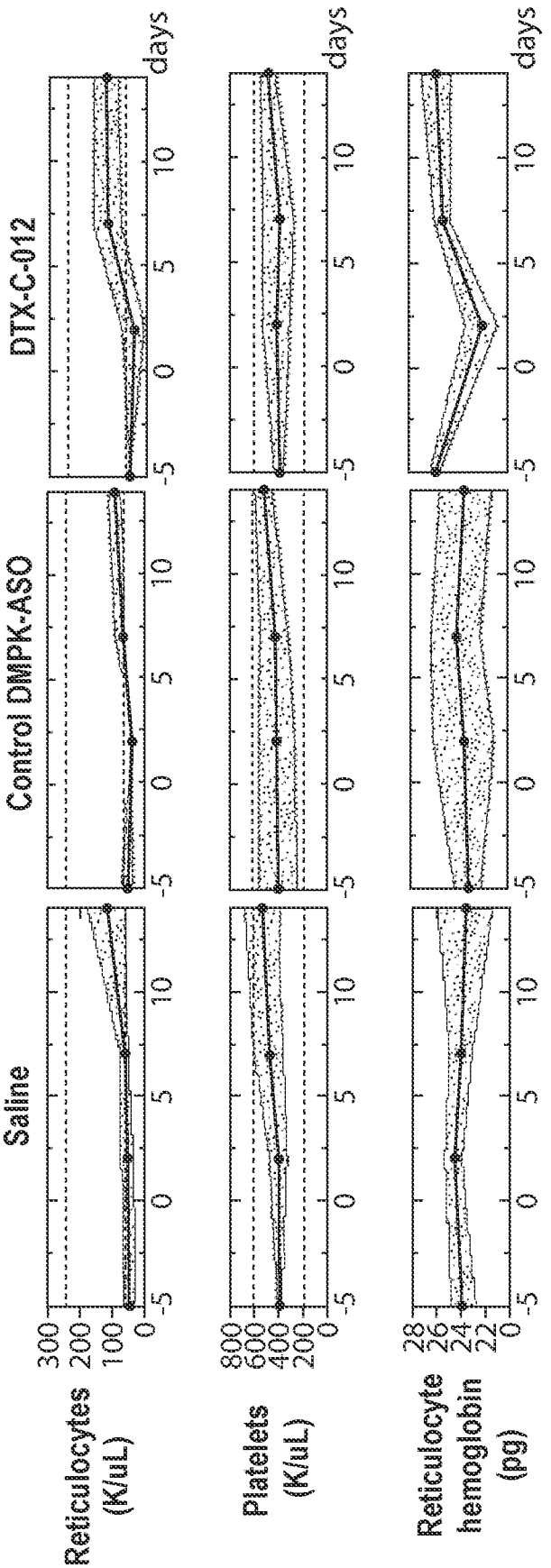
Saline
Control DMPK-ASO
DTX-C-012
Reticulocytes (K/uL)
Platelets (K/uL)
Reticulocyte hemoglobin (pg)
days
300
200
100
0
800
600
400
200
28
26
24
22
-5
0
5
10

FIG. 21