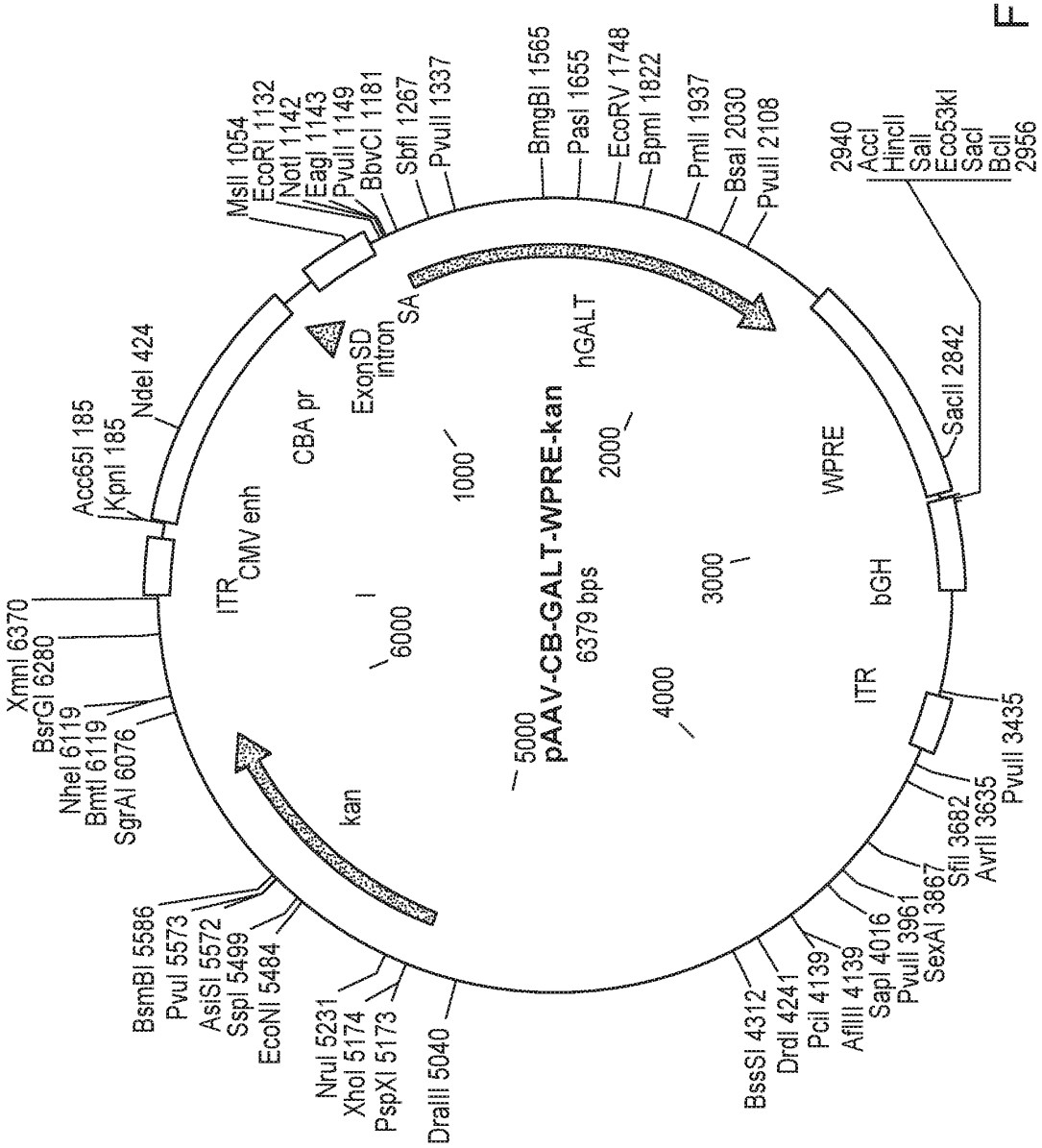

pAAV-CB-GALT-WPRE-kan
6379 bps
1000
2000
3000
4000
5000
6000
ITR
CMV enh
CBA pr
ExonSD
intron
SA
hGALT
WPRE
bGH
ITR
kan
MslI 1054
EcoRI 1132
NotI 1142
EagI 1143
PvuII 1149
BbvCI 1181
SbfI 1267
PvuII 1337
BmgBI 1565
PasI 1655
EcoRV 1748
BpmI 1822
PmlI 1937
BsaI 2030
PvuII 2108
2940
AccI
HincII
SalI
Eco53kI
SacI
BclI
2956
SacII 2842
PvuII 3435
SfiI 3682
AvrII 3635
SexAI 3867
PvuII 3961
SapI 4016
AflIII 4139
PciI 4139
DrdI 4241
BssSI 4312
DraIII 5040
XhoI 5174
PspXI 5173
NruI 5231
EcoNI 5484
SspI 5499
AsiSI 5572
PvuI 5573
BsmBI 5586
SgrAI 6076
BmtI 6119
NheI 6119
BsrGI 6280
XmnI 6370
Acc65I 185
KpnI 185
NdeI 424

FIG. 1A

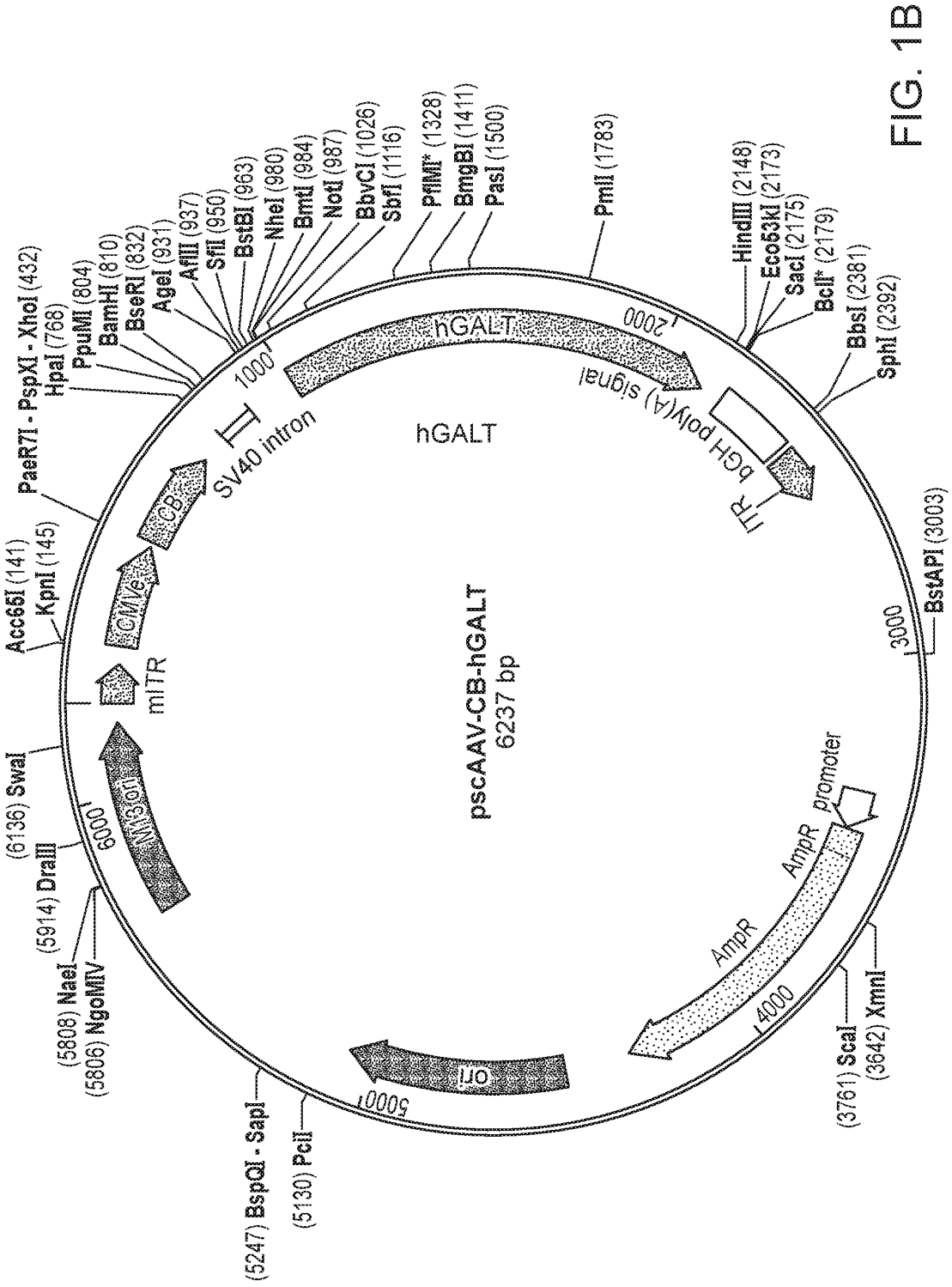

pscAAV-CB-hGALT
6237 bp
hGALT
hGALT
SV40 intron
CB
CMVe
mITR
M13 ori
ITR
bGH polyA signal
AmpR
promoter
ori
1000
2000
3000
4000
5000
6000
Acc65I (141)
KpnI (145)
PaeR7I - PspXI - XhoI (432)
HpaI (768)
PpuMI (804)
BamHI (810)
BseRI (832)
AgeI (931)
AflII (937)
SfiI (950)
BstBI (963)
NheI (980)
BmtI (984)
NotI (987)
BbvCI (1026)
SbfI (1116)
PflMI* (1328)
BmgBI (1411)
PasI (1500)
PmlI (1783)
HindIII (2148)
Eco53kI (2173)
SacI (2175)
BclI* (2179)
BbsI (2381)
SphI (2392)
BstAPI (3003)
XmnI (3642)
ScaI (3761)
(5130) PciI
(5247) BspQI - SapI
(5806) NgoMIV
(5808) NaeI
(5914) DraIII
(6136) SwaI

FIG. 1B

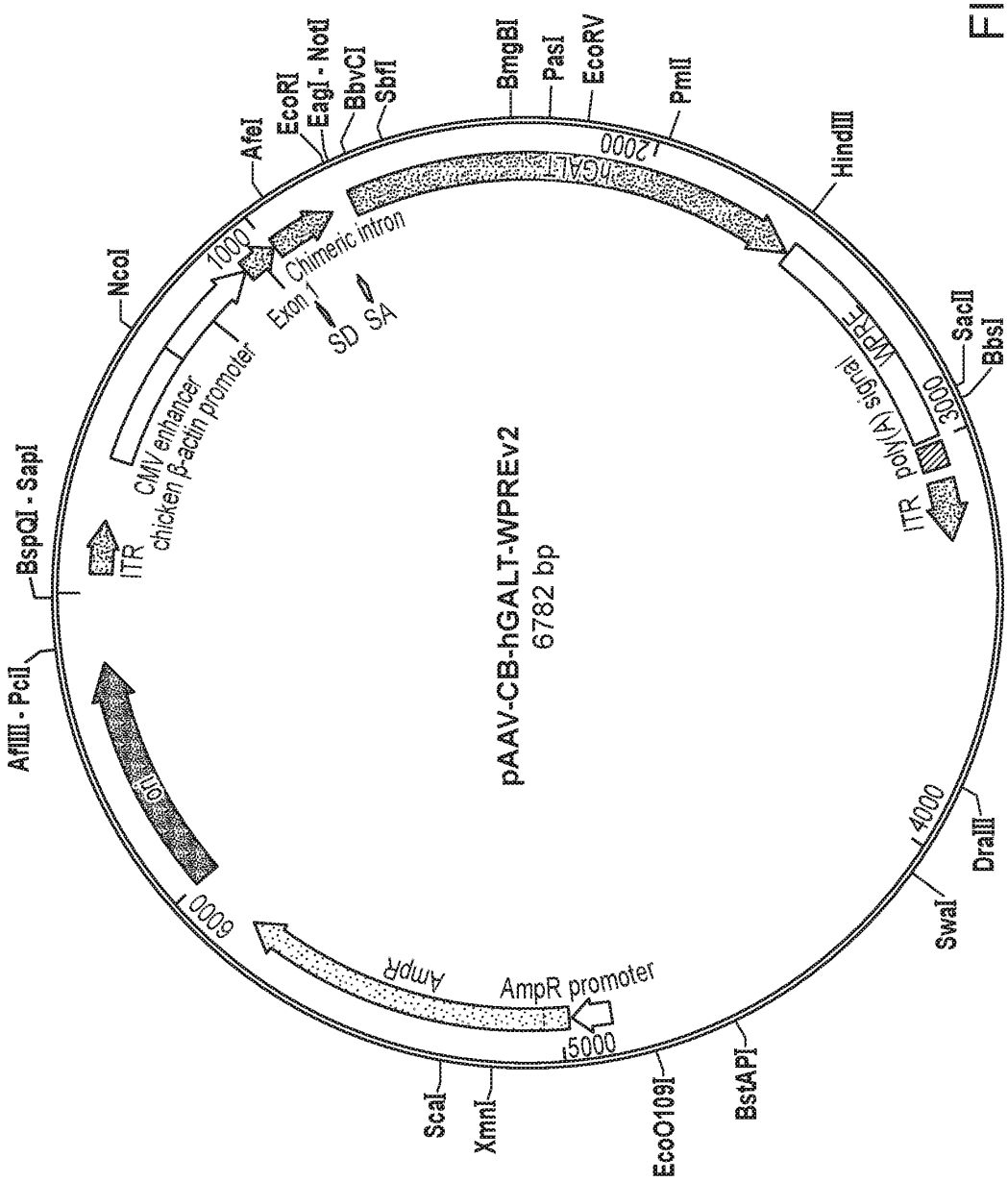

pAAV-CB-hGALT-WPREv2
6782 bp
hGALT
WPRE
ITR
poly(A) signal
AmpR
AmpR promoter
ori
CMV enhancer
chicken β-actin promoter
Chimeric intron
Exon 1
SD
SA
1000
2000
3000
4000
5000
6000
AflII
EcoRI
EagI - NotI
BbvCI
SbfI
BmgBI
PasI
EcoRV
PmlI
HindIII
SacII
BbsI
DraIII
SwaI
BstAPI
EcoO109I
XmnI
ScaI
AflIII - PciI
BspQI - SapI
NcoI

FIG. 1C

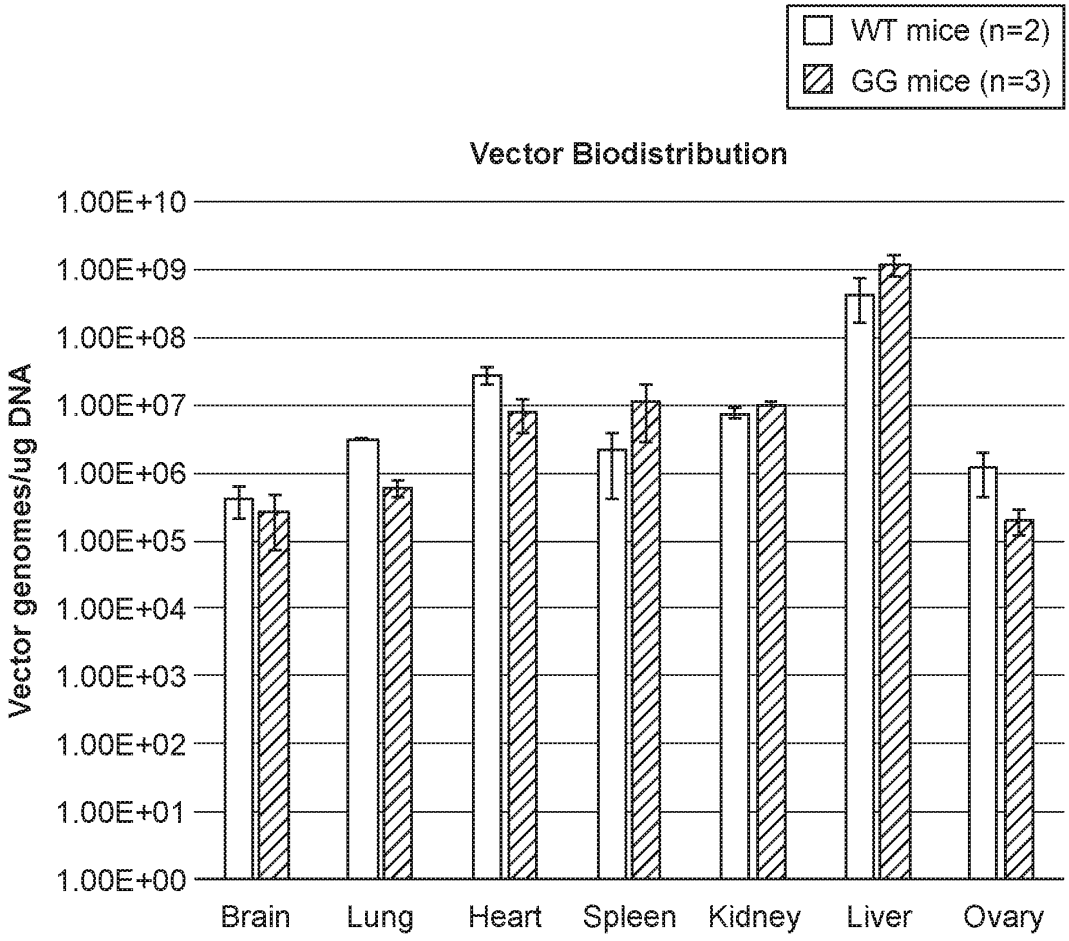

WT mice (n=2)
GG mice (n=3)
Vector Biodistribution
Vector genomes/ug DNA
1.00E+10
1.00E+09
1.00E+08
1.00E+07
1.00E+06
1.00E+05
1.00E+04
1.00E+03
1.00E+02
1.00E+01
1.00E+00
Brain
Lung
Heart
Spleen
Kidney
Liver
Ovary

FIG. 2

									Dilutions	
kDa	Marker	1ul GALT lysate (29.78ug)	0.1ul GALT lysate (2.98ug)	5ul lucEYFP control lysate (137.63ug)	Empty	HEPG2 cell lysate (135ug)	Empty	1ul + cntrl (0.5ug)	5ul + ctrl (2.5ug)	10ul + ctrl (5.0ug)
260										
125										
90										
70										
50										
38										
30										
25										
8										

Predicted GALT MW= 43.2 kDa

Marker: Chameleon Duo-Prestained Protein Ladder

Dilutions were made for the GALT from = GALT control protein + lysate ctrl + 2x SDS

Starting concentration of + control of GALT proteins is 100 ug/200ul

Loaded cell lysates into the wells (ul)		Total protein concentrations(mg/ml)	
GALT	1	GALT	29.779
GALT(10X diluted)	1	lucEYFP	27.526
lucEYFP	5	HEPG2	4.16
HEPG2	32.5		

FIG. 3

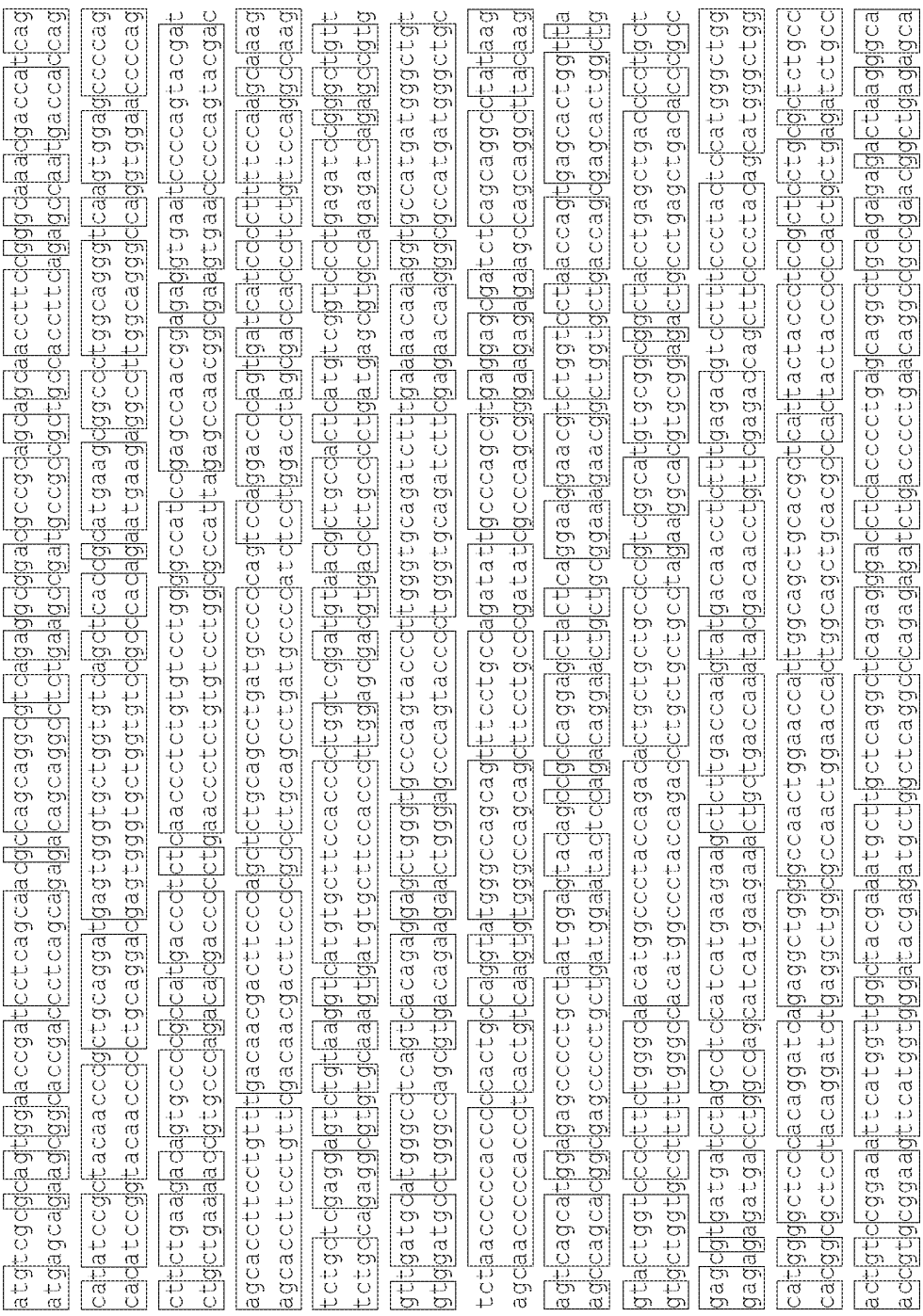


FIG. 4

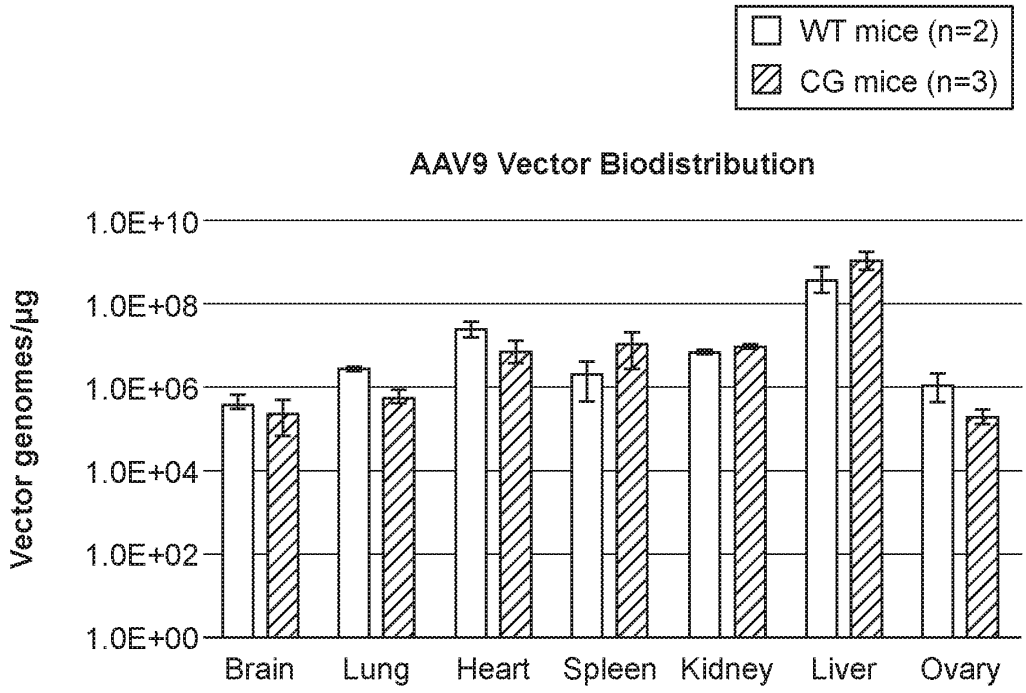

WT mice (n=2)
CG mice (n=3)
AAV9 Vector Biodistribution
Vector genomes/µg
1.0E+10
1.0E+08
1.0E+06
1.0E+04
1.0E+02
1.0E+00
Brain
Lung
Heart
Spleen
Kidney
Liver
Ovary

FIG. 5

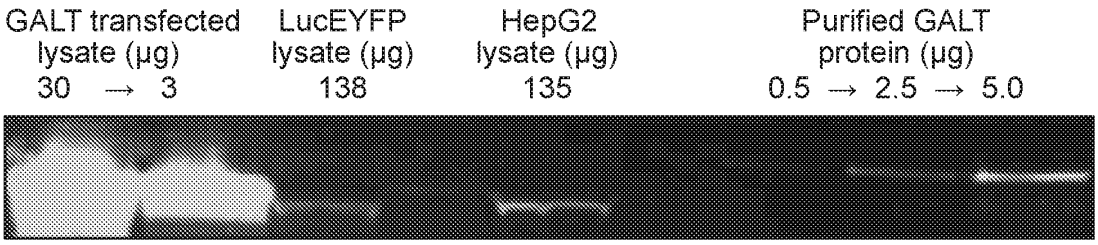

GALT transfected lysate (µg)
30 → 3
LucEYFP lysate (µg)
138
HepG2 lysate (µg)
135
Purified GALT protein (µg)
0.5 → 2.5 → 5.0

FIG. 6