Figure 1

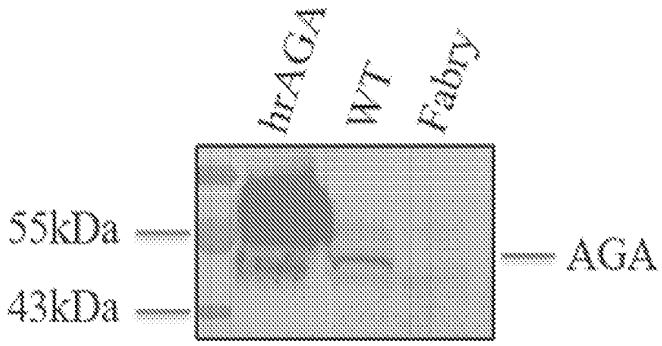


Figure 2A

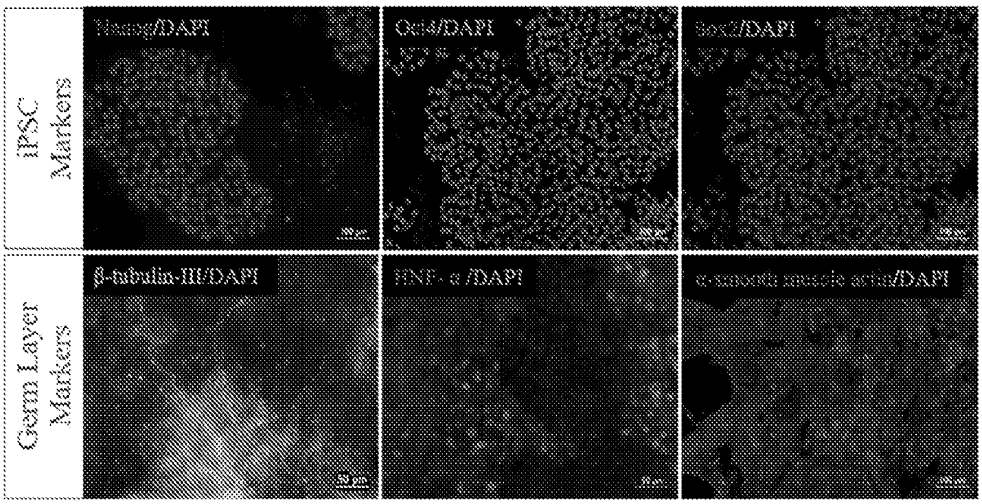
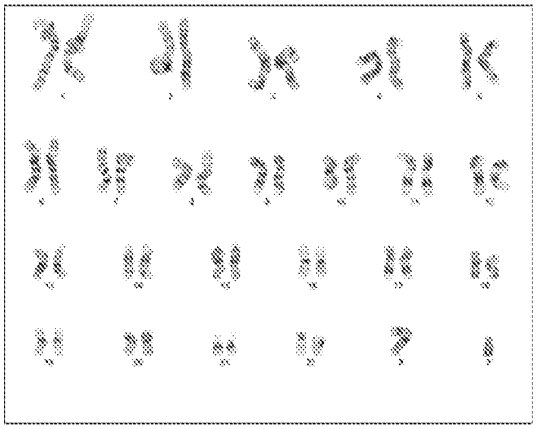


Figure 2B



Banding Technique: GTL Band Resolution: Good
Metaphases Counted: 20 Analyzed: 7 Karyotyped: 2

RESULTS: 46,XY[20] Apparently NORMAL Human Male Karyotype

Non-clonal Aberrations: None

Figure 3A

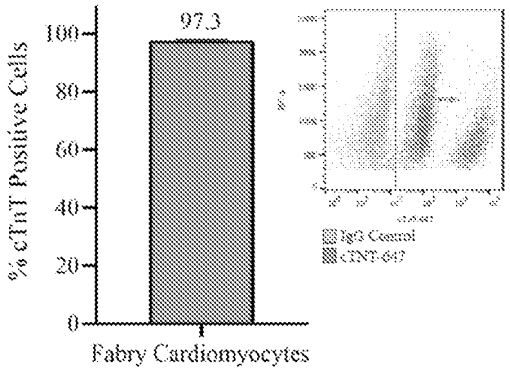


Figure 3B

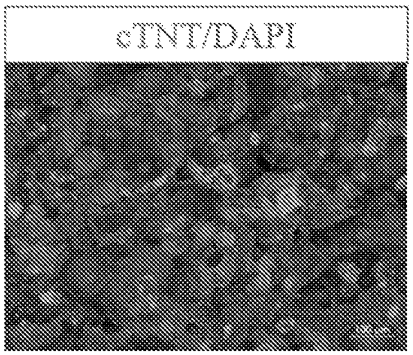


Figure 4A

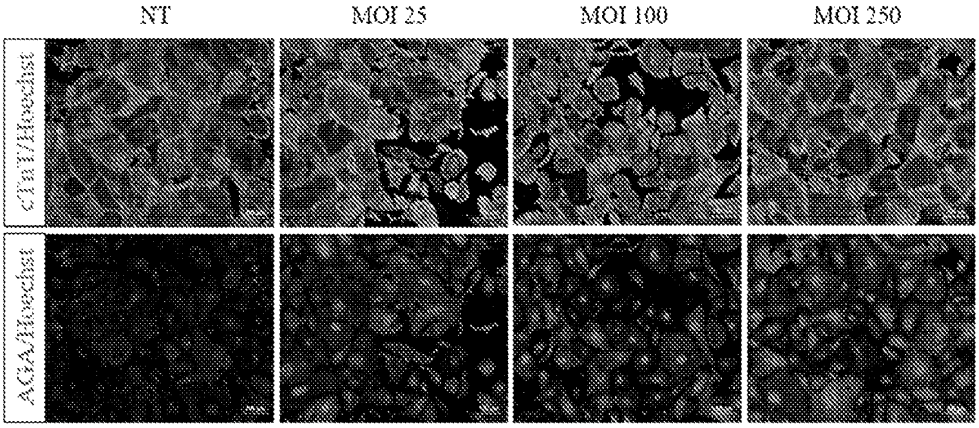


Figure 4B

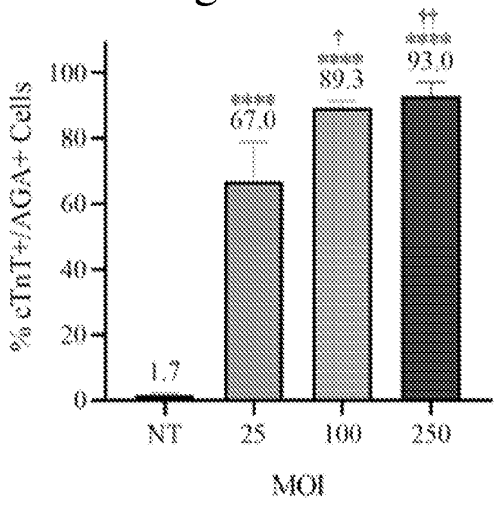


Figure 4C

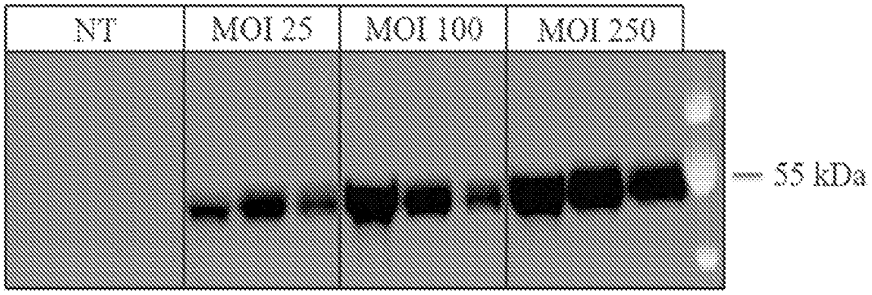


Figure 5A

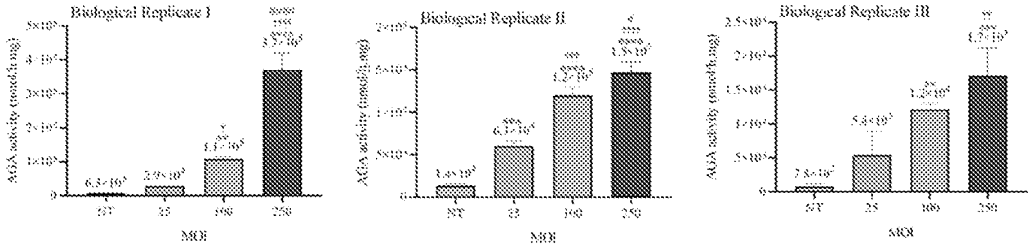


Figure 5B

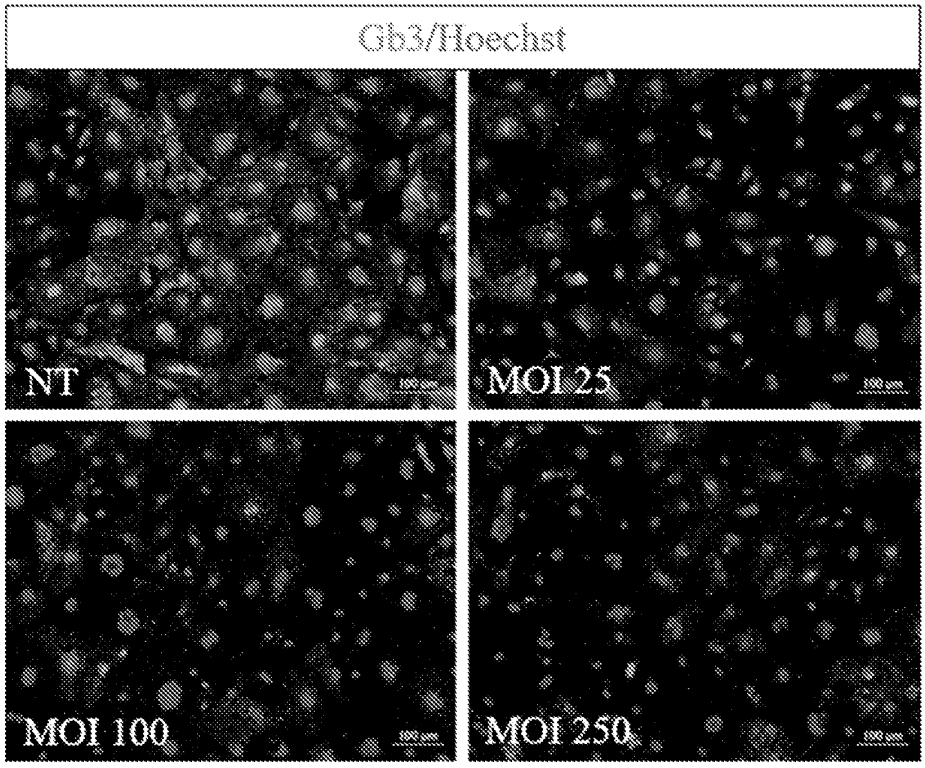


Figure 6A

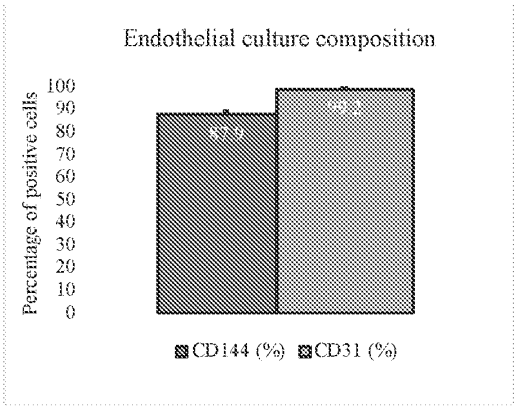


Figure 6B

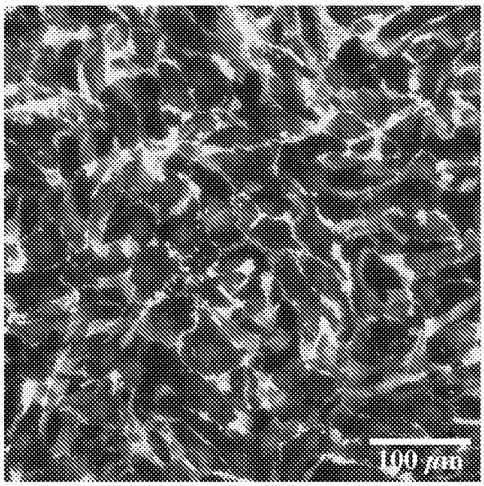


Figure 7A

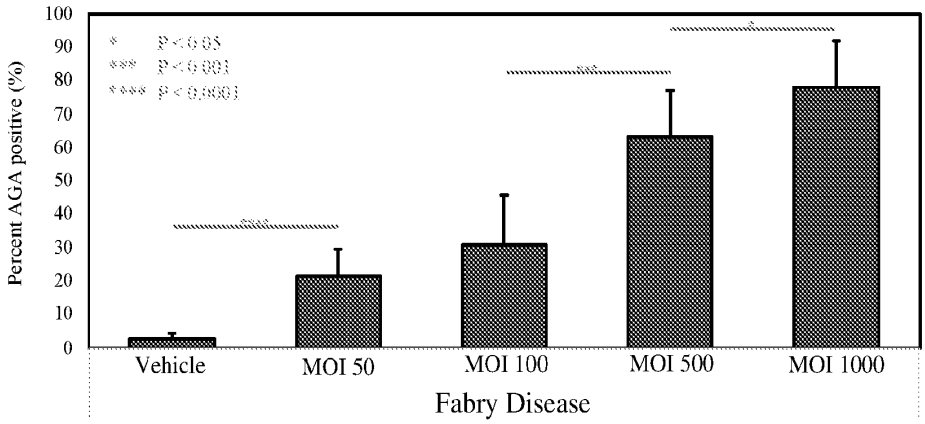
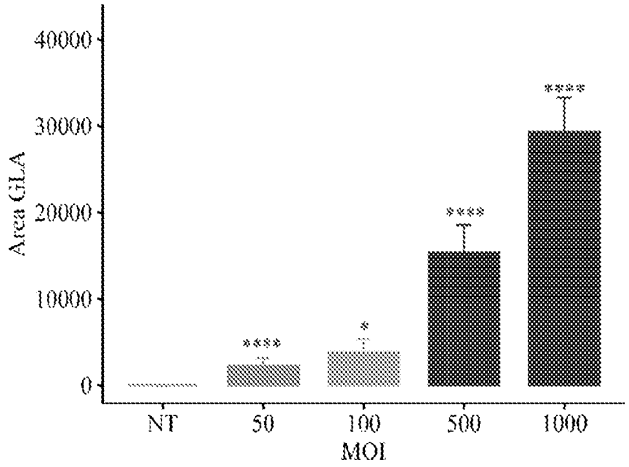


Figure 7B



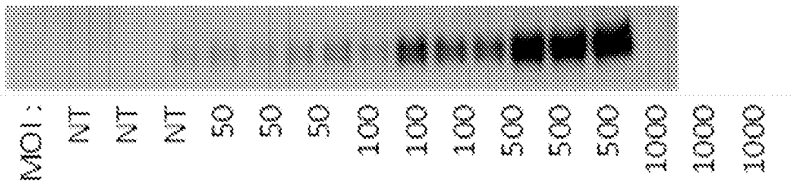


Figure 8A

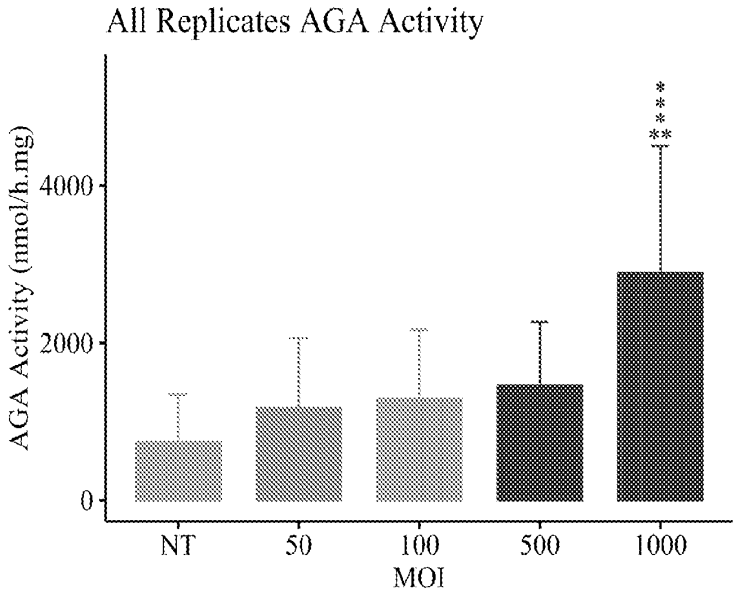


Figure 8B

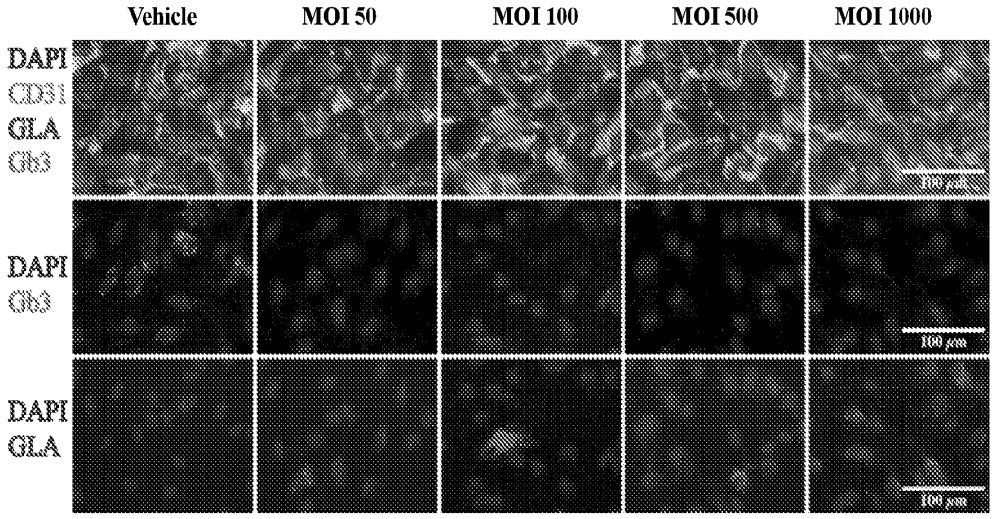


Figure 9A

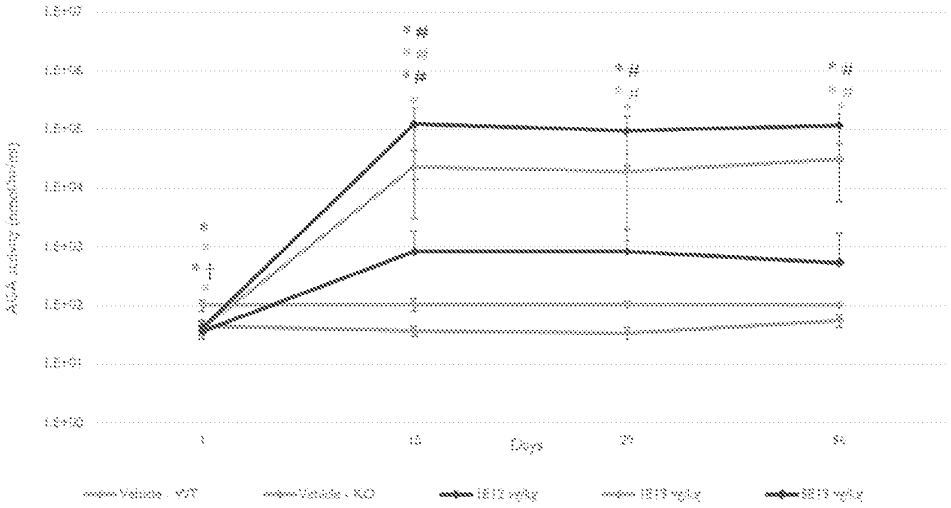


Figure 9B

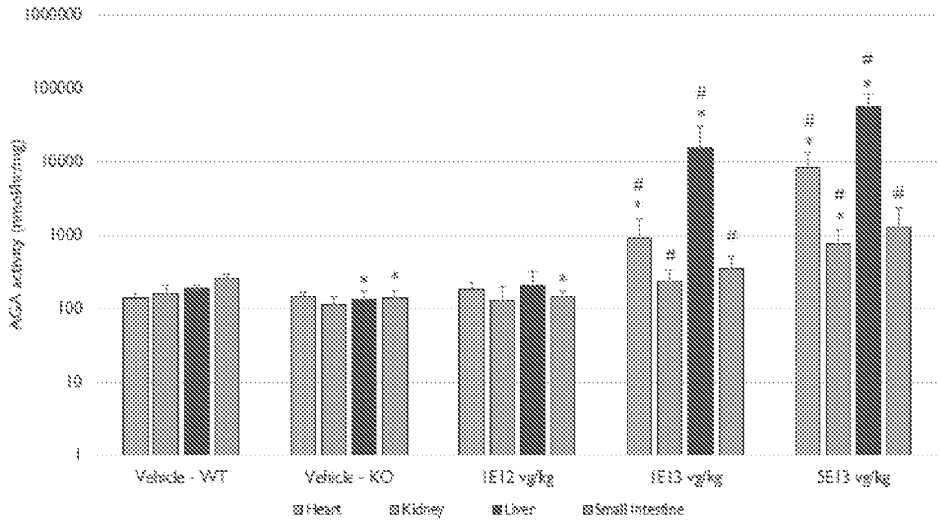


Figure 10

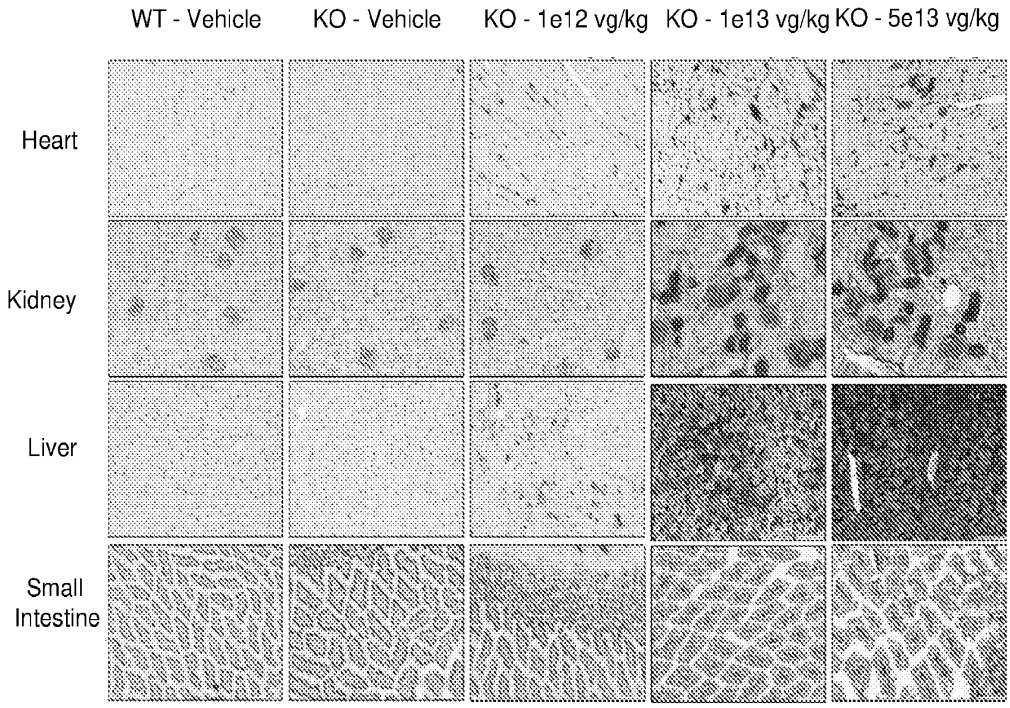

WT - Vehicle
KO - Vehicle
KO - 1e12 vg/kg
KO - 1e13 vg/kg
KO - 5e13 vg/kg
Heart
Kidney
Liver
Small Intestine

Figure 11

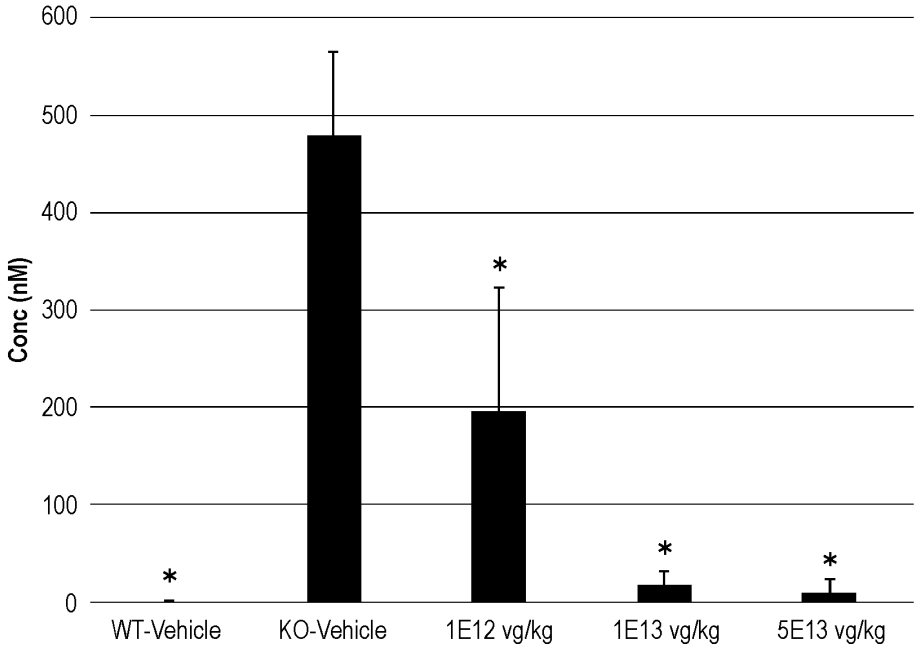

600
500
400
300
200
100
0
Conc (nM)
*
*
*
*
WT-Vehicle
KO-Vehicle
1E12 vg/kg
1E13 vg/kg
5E13 vg/kg

Figure 12

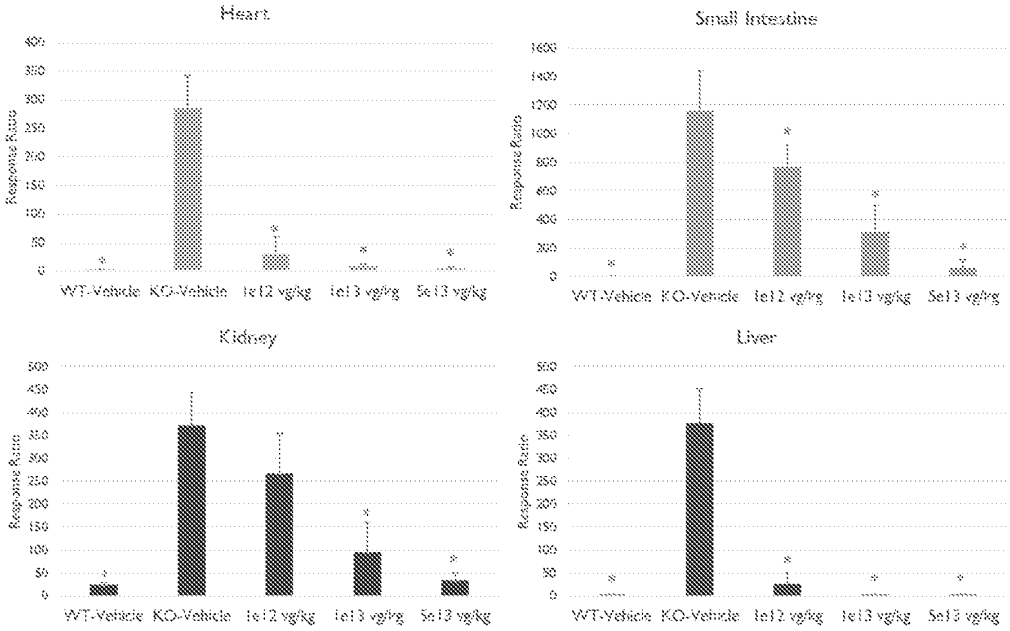
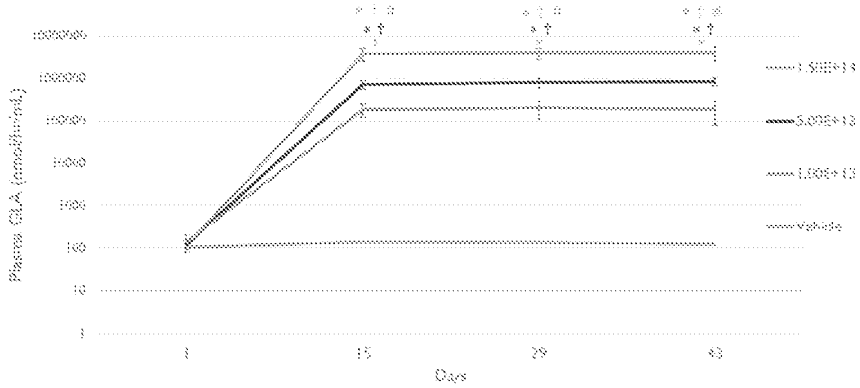

Heart
Small Intestine
Kidney
Liver
Response Ratio
WT-Vehicle
KO-Vehicle
1e12 vg/kg
1e13 vg/kg
5e13 vg/kg

Figure 13



Dose (vg/kg)	Fold Change GLA Values
1e13	~1200
5e13	~5000
1.5e14	~26,000

Figure 14

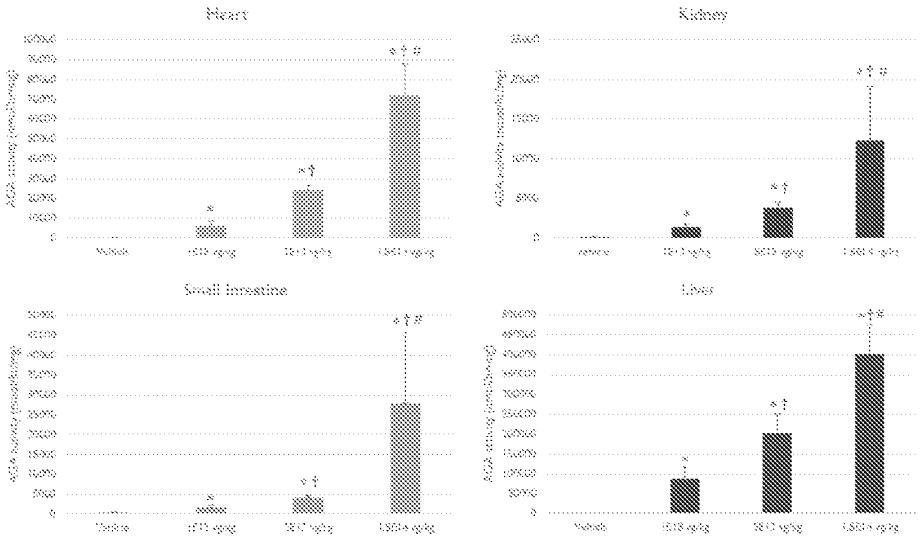


Figure 15

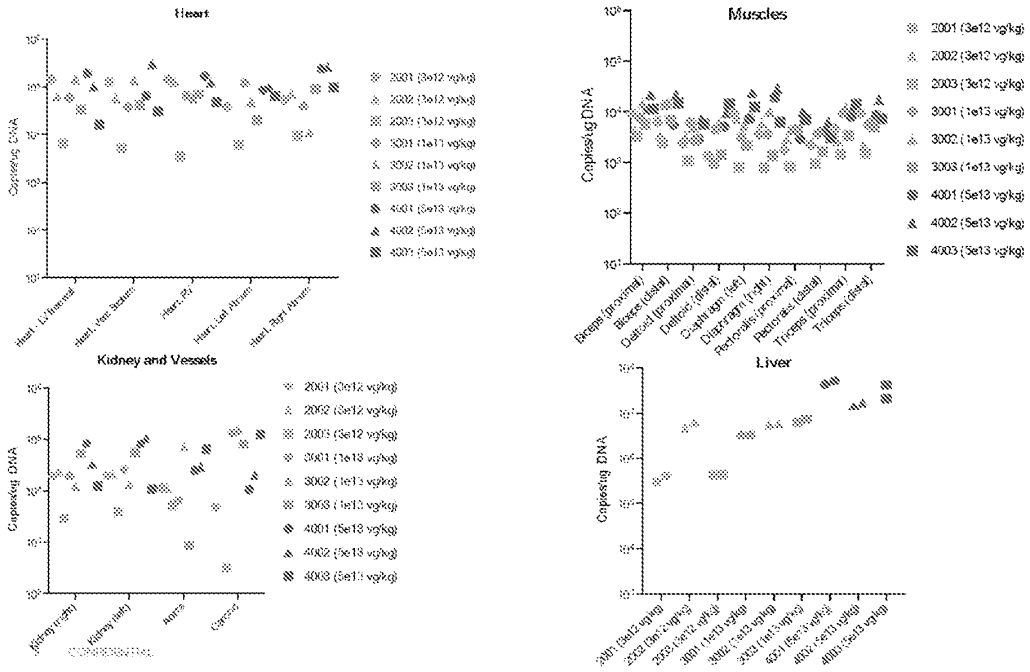
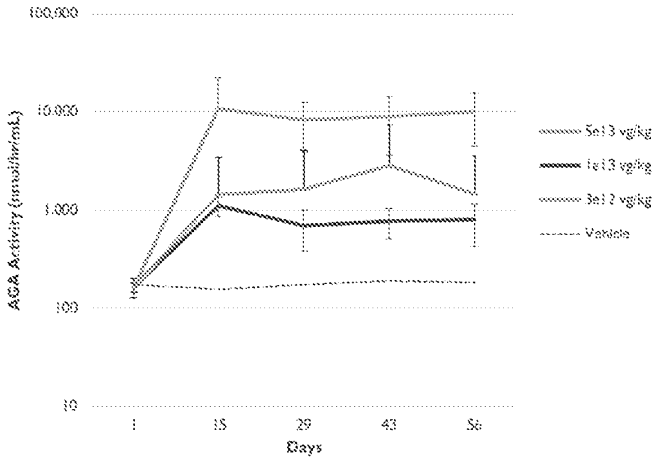

Heart
Copies/ug DNA
2001 (3e12 vg/kg)
2002 (3e12 vg/kg)
2003 (3e12 vg/kg)
3001 (1e13 vg/kg)
3002 (1e13 vg/kg)
3003 (1e13 vg/kg)
4001 (5e13 vg/kg)
4002 (5e13 vg/kg)
4003 (5e13 vg/kg)
Heart, LV lateral
Heart, Ant Septum
Heart, RV
Heart, Lat Atrium
Heart, Sept Atrium
Muscles
Copies/ug DNA
Biceps (proximal)
Biceps (distal)
Deltoid (proximal)
Deltoid (distal)
Diaphragm (left)
Diaphragm (right)
Pectoralis (proximal)
Pectoralis (distal)
Triceps (proximal)
Triceps (distal)
Kidney and Vessels
Copies/ug DNA
Kidney (right)
Kidney (left)
Aorta
Carotid
Liver
Copies/ug DNA

Figure 16

Plasma AGA – Dose Group Means



Dose (vg/kg)	Fold Increase Relative to Day 1
3e12	7.9
1e13	4.3
5e13	54.7

Figure 17

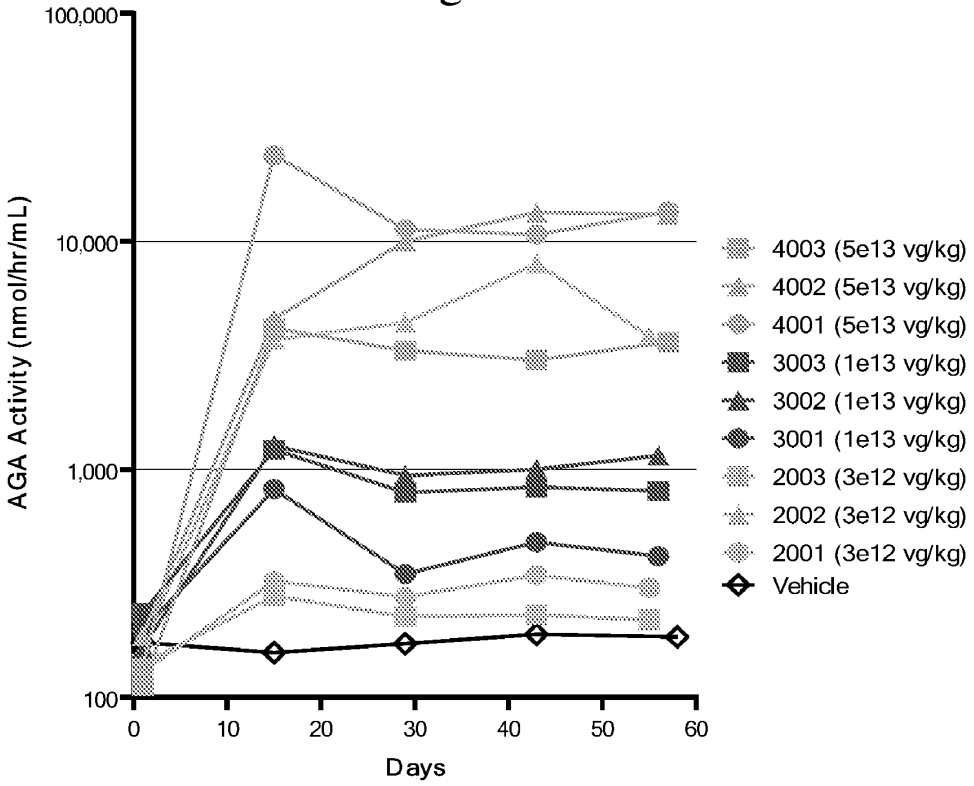

100,000
10,000
1,000
100
AGA Activity (nmol/hr/mL)
0
10
20
30
40
50
60
Days
4003 (5e13 vg/kg)
4002 (5e13 vg/kg)
4001 (5e13 vg/kg)
3003 (1e13 vg/kg)
3002 (1e13 vg/kg)
3001 (1e13 vg/kg)
2003 (3e12 vg/kg)
2002 (3e12 vg/kg)
2001 (3e12 vg/kg)
Vehicle

Figure 18

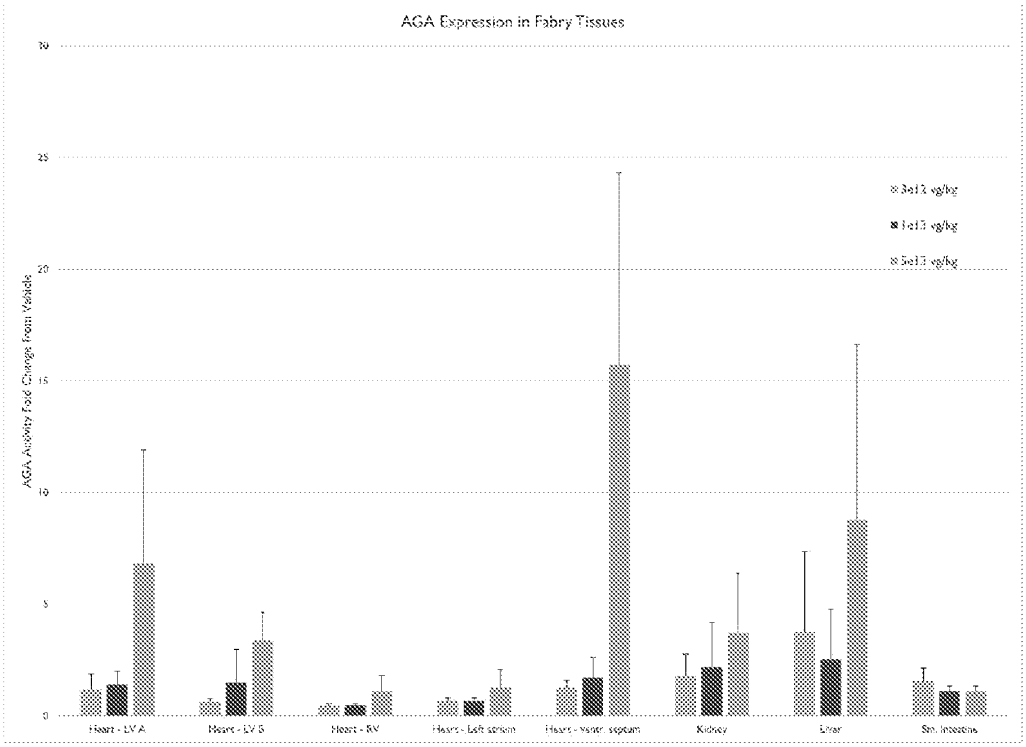
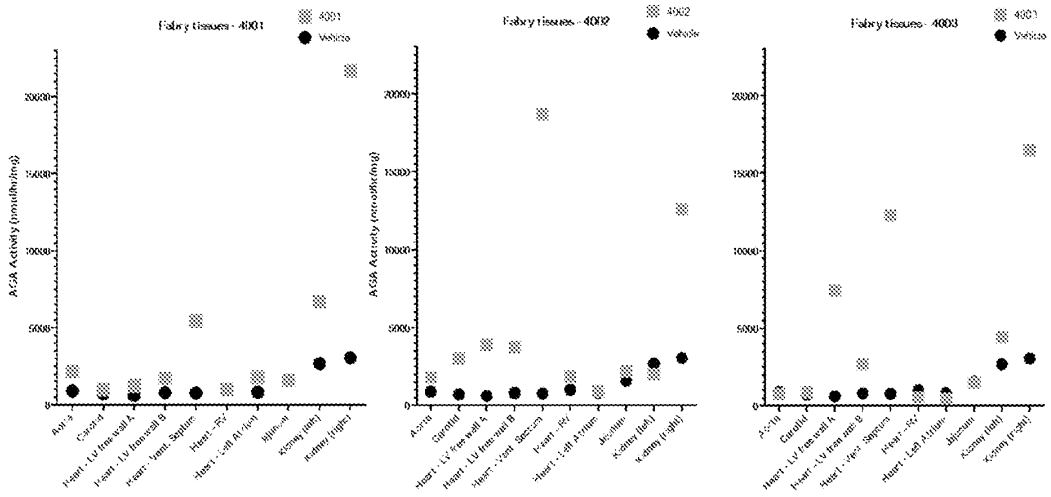

AGA Expression in Fabry Tissues
AGA Activity Fold Change From Vehicle
30
25
20
15
10
5
0
3e12 vg/kg
1e13 vg/kg
3e13 vg/kg
Heart - LV A
Heart - LV S
Heart - RV
Heart - Left septum
Heart - Inter. septum
Kidney
Liver
Sm. Intestine

Figure 19


Fabry tissues - 4001
4001
Vehicle
AGA Activity (nmol/hr/mg)
Fabry tissues - 4002
4002
Vehicle
AGA Activity (nmol/hr/mg)
Fabry tissues - 4003
4003
Vehicle

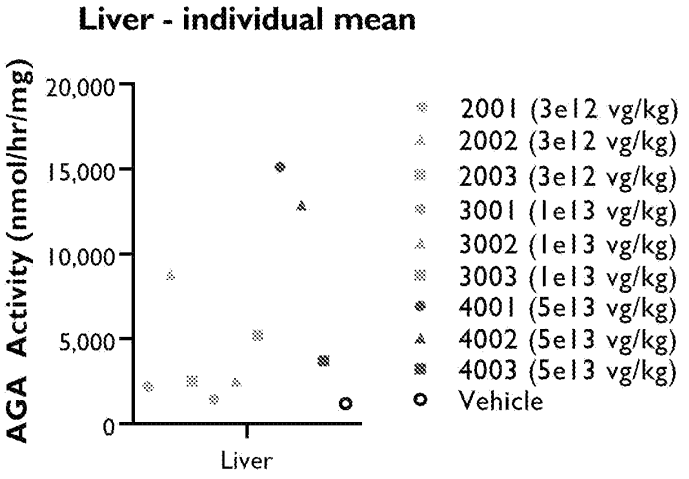

Liver - individual mean
AGA Activity (nmol/hr/mg)
20,000
15,000
10,000
5,000
0
Liver
2001 (3e12 vg/kg)
2002 (3e12 vg/kg)
2003 (3e12 vg/kg)
3001 (1e13 vg/kg)
3002 (1e13 vg/kg)
3003 (1e13 vg/kg)
4001 (5e13 vg/kg)
4002 (5e13 vg/kg)
4003 (5e13 vg/kg)
Vehicle

Figure 20

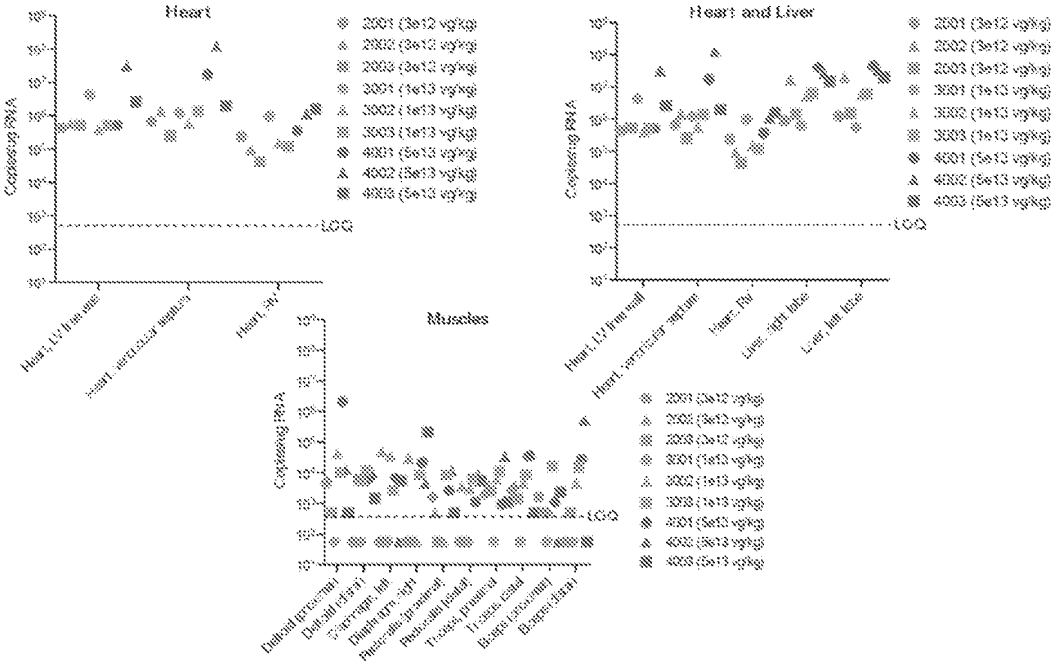

Heart
Heart and Liver
Muscles
Copies/µg RNA
LOQ
2001 (3e12 vg/kg)
2002 (3e12 vg/kg)
2003 (3e12 vg/kg)
3001 (1e13 vg/kg)
3002 (1e13 vg/kg)
3003 (1e13 vg/kg)
4001 (5e13 vg/kg)
4002 (5e13 vg/kg)
4003 (5e13 vg/kg)

Figure 21A

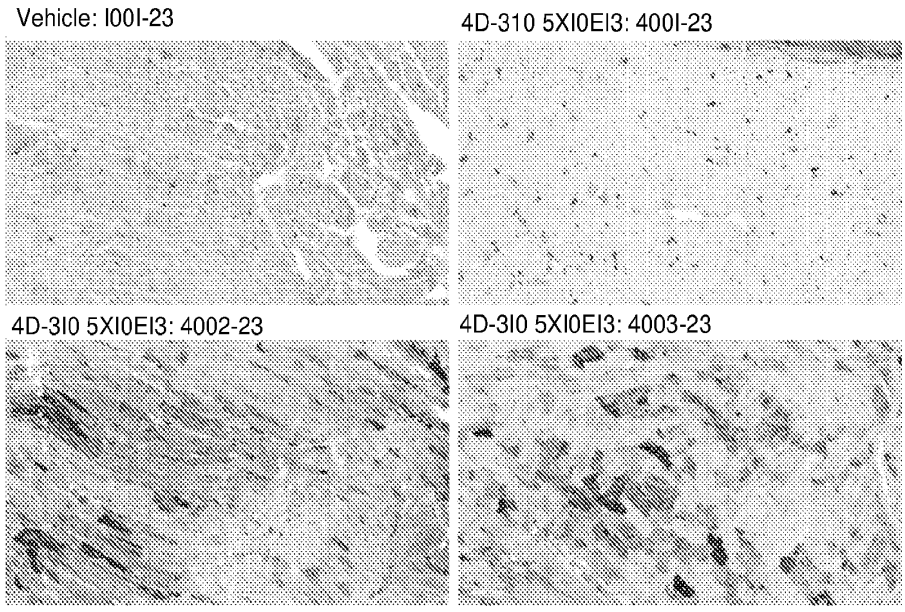


Figure 21B

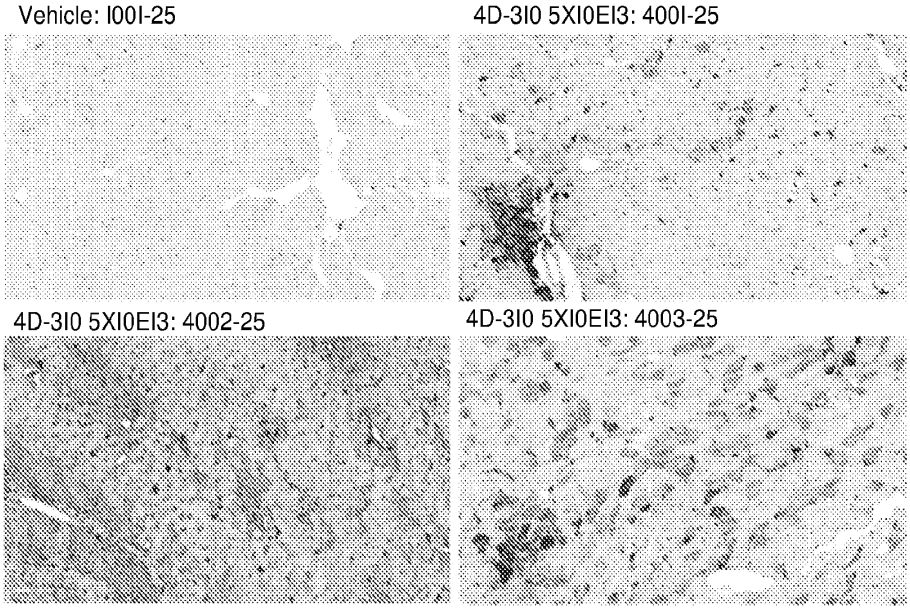


Figure 22A

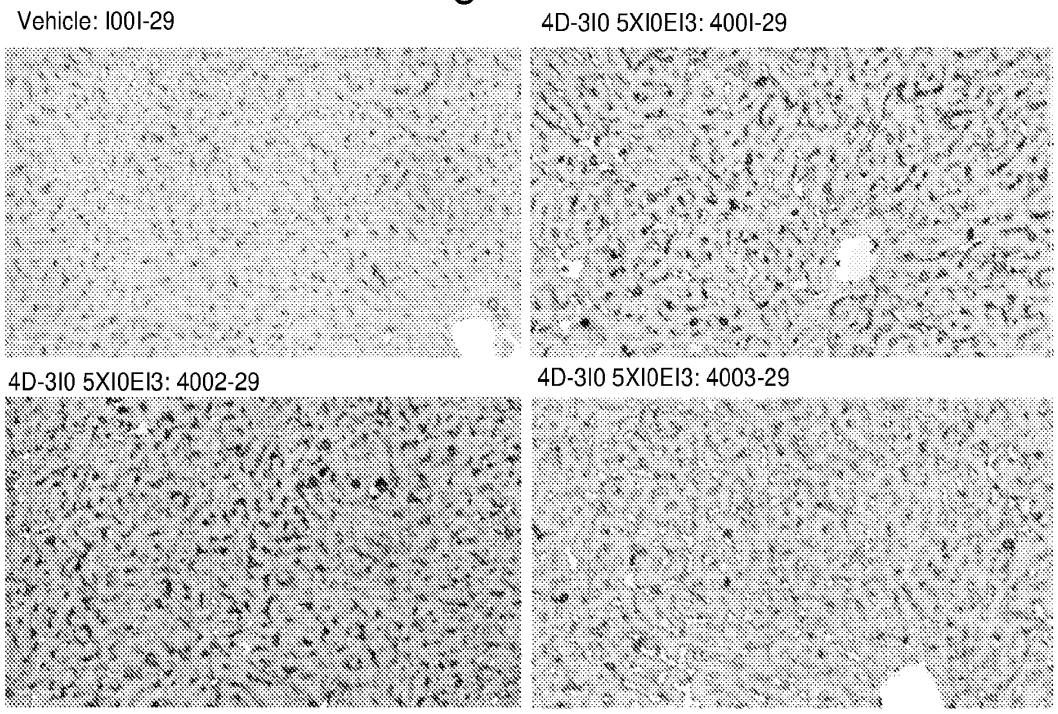


Figure 22B

