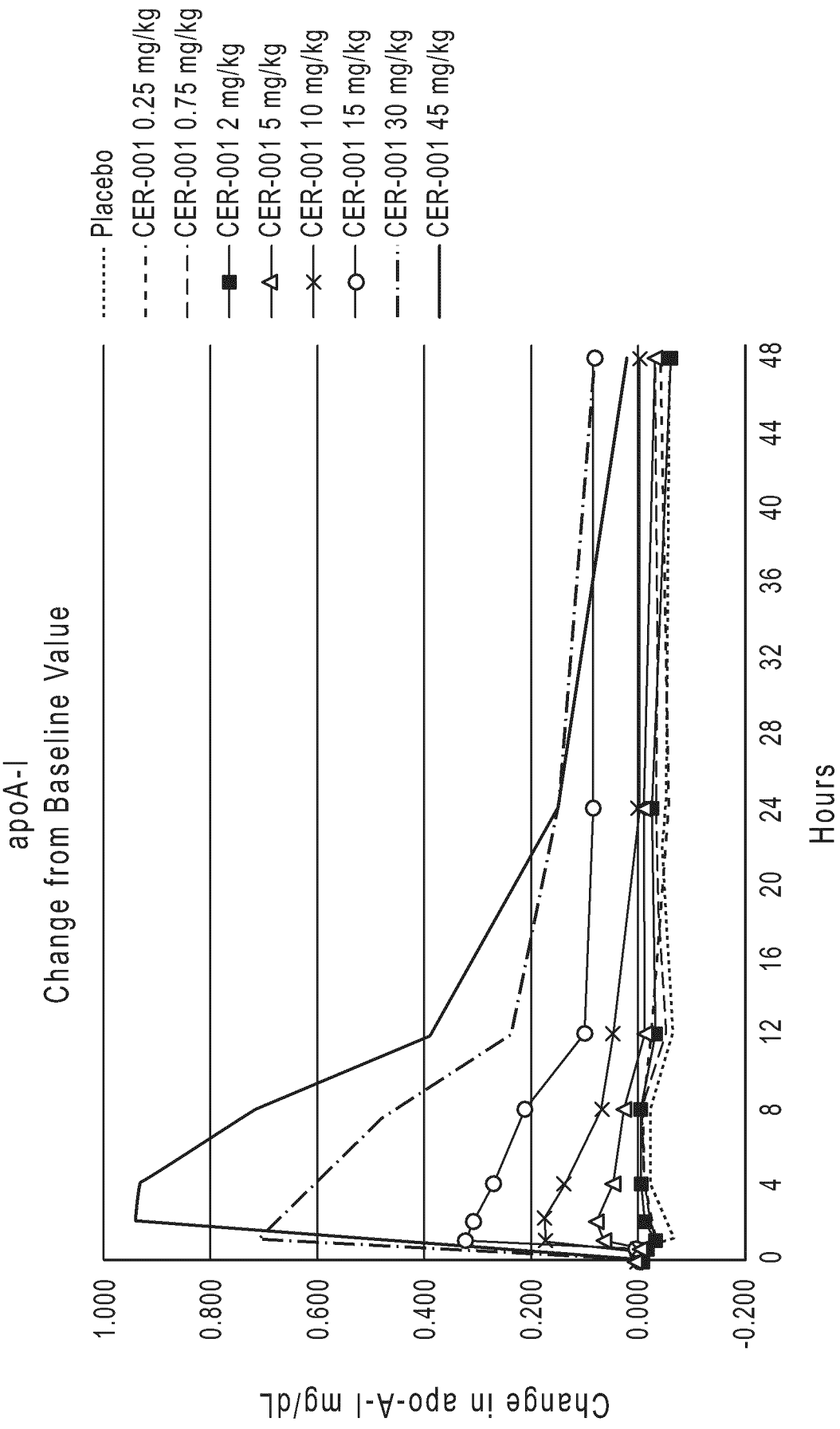

apoA-I
Change from Baseline Value
Placebo
CER-001 0.25 mg/kg
CER-001 0.75 mg/kg
CER-001 2 mg/kg
CER-001 5 mg/kg
CER-001 10 mg/kg
CER-001 15 mg/kg
CER-001 30 mg/kg
CER-001 45 mg/kg
1.000
0.800
0.600
0.400
0.200
0.000
-0.200
0
4
8
12
16
20
24
28
32
36
40
44
48
Hours
Change in apo-A-I mg/dL

Fig. 1

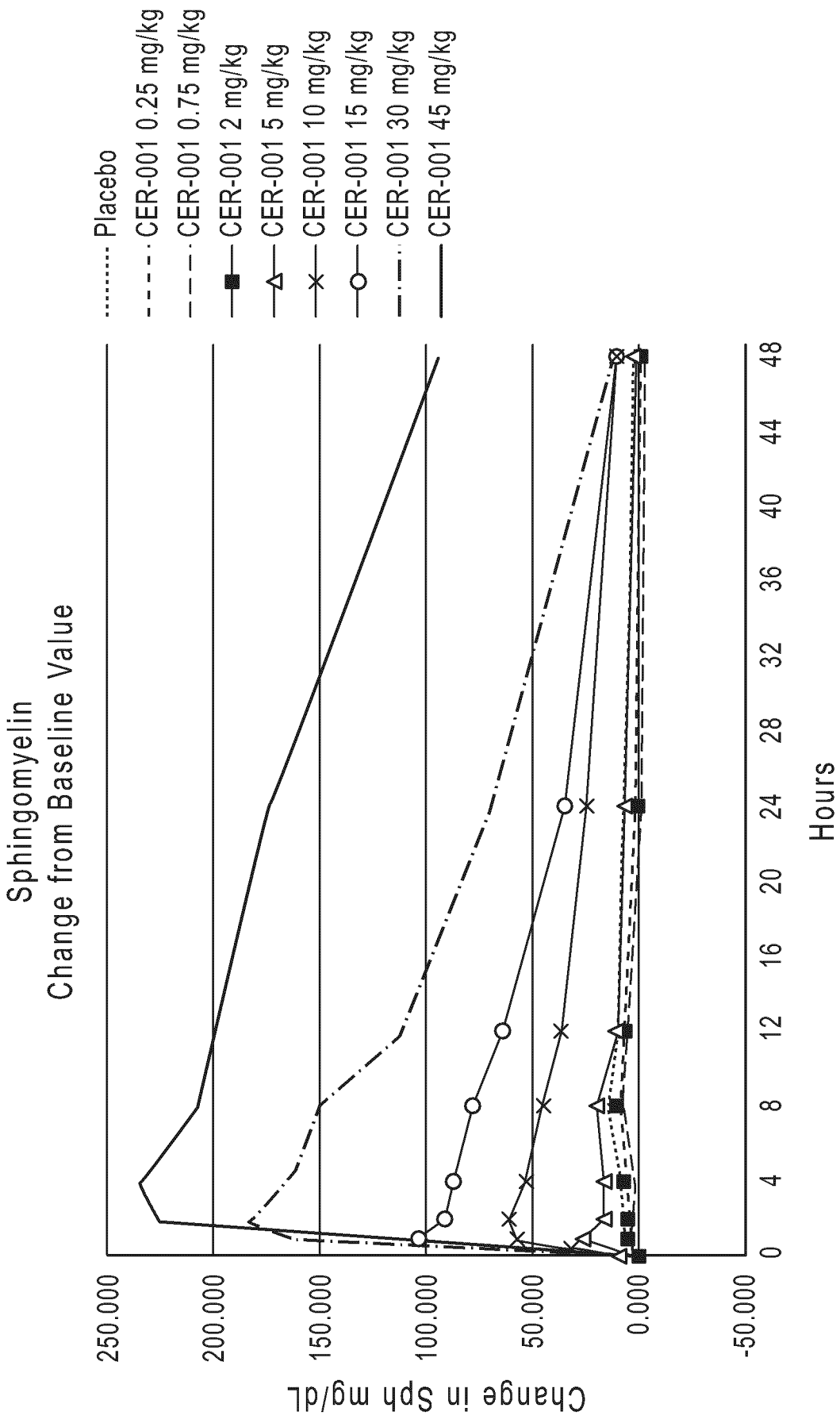

Sphingomyelin
Change from Baseline Value
Placebo
CER-001 0.25 mg/kg
CER-001 0.75 mg/kg
CER-001 2 mg/kg
CER-001 5 mg/kg
CER-001 10 mg/kg
CER-001 15 mg/kg
CER-001 30 mg/kg
CER-001 45 mg/kg
Change in Sph mg/dL
250.000
200.000
150.000
100.000
50.000
0.000
-50.000
0
4
8
12
16
20
24
28
32
36
40
44
48
Hours

Fig. 2

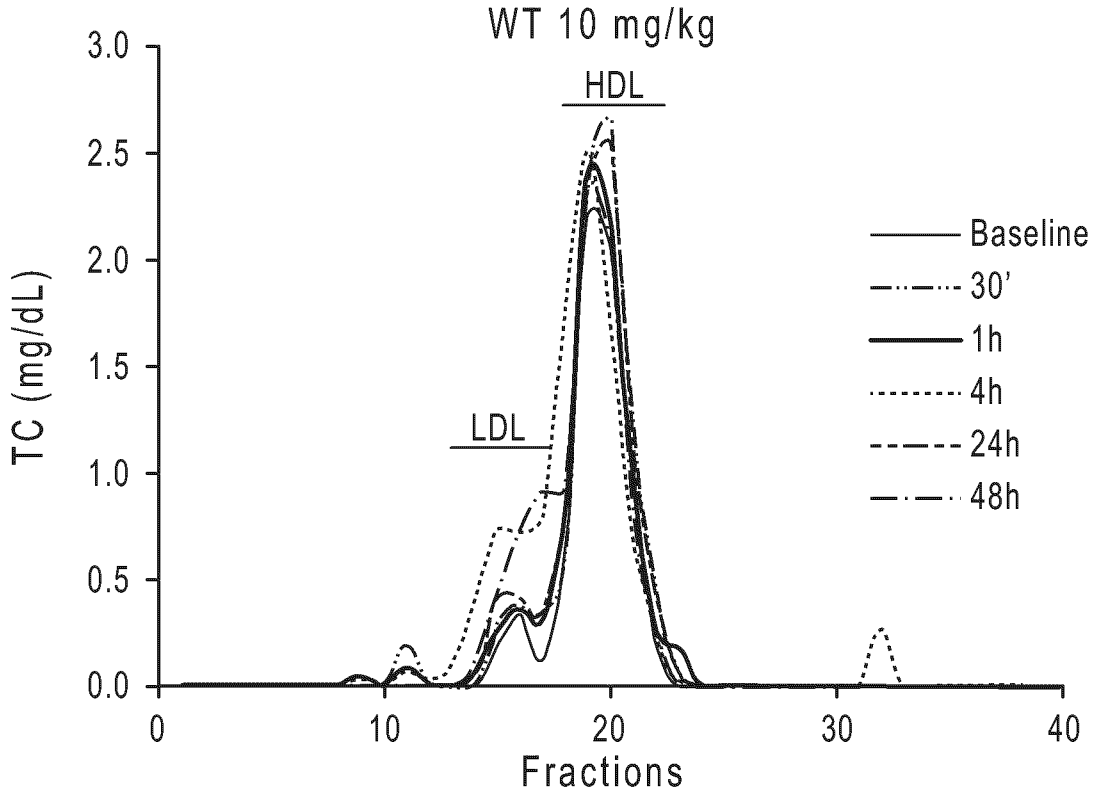

WT 10 mg/kg
HDL
LDL
TC (mg/dL)
Fractions
Baseline
30'
1h
4h
24h
48h
0.0
0.5
1.0
1.5
2.0
2.5
3.0
0
10
20
30
40

Fig. 3A

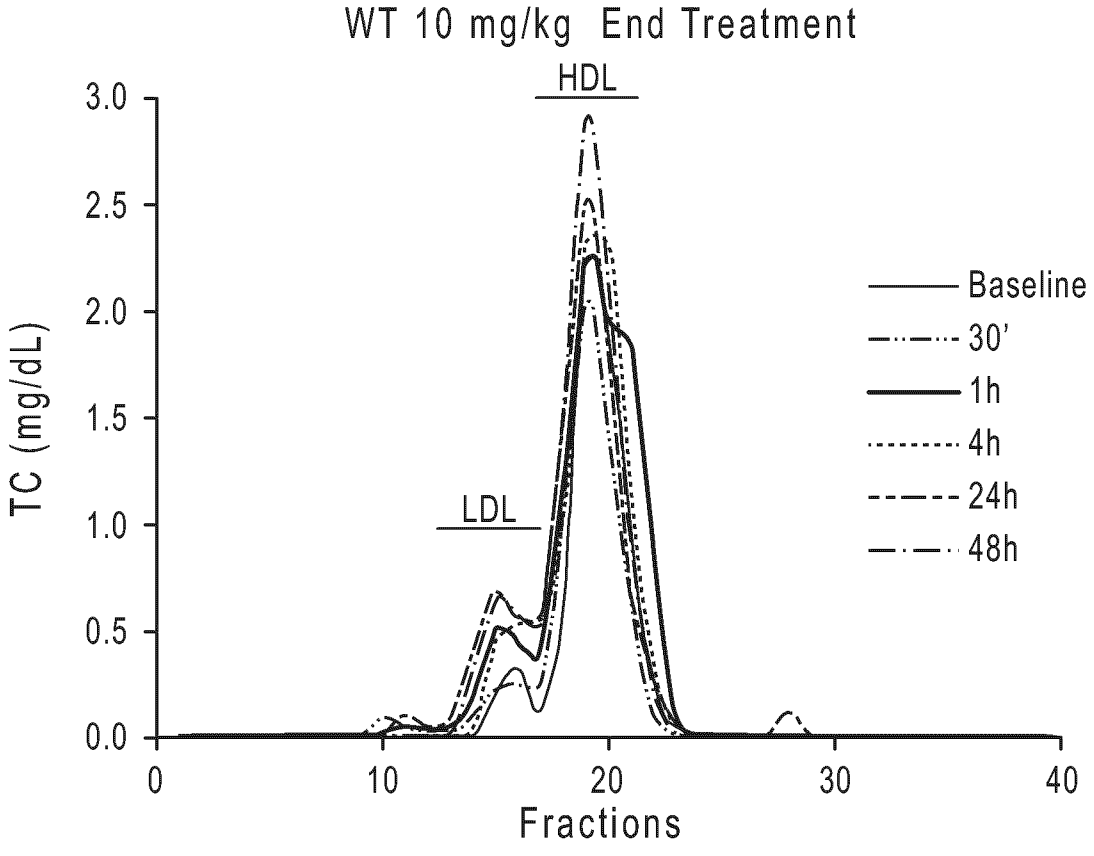

WT 10 mg/kg End Treatment
HDL
LDL
TC (mg/dL)
Fractions
Baseline
30'
1h
4h
24h
48h
0.0
0.5
1.0
1.5
2.0
2.5
3.0
0
10
20
30
40

Fig. 3B

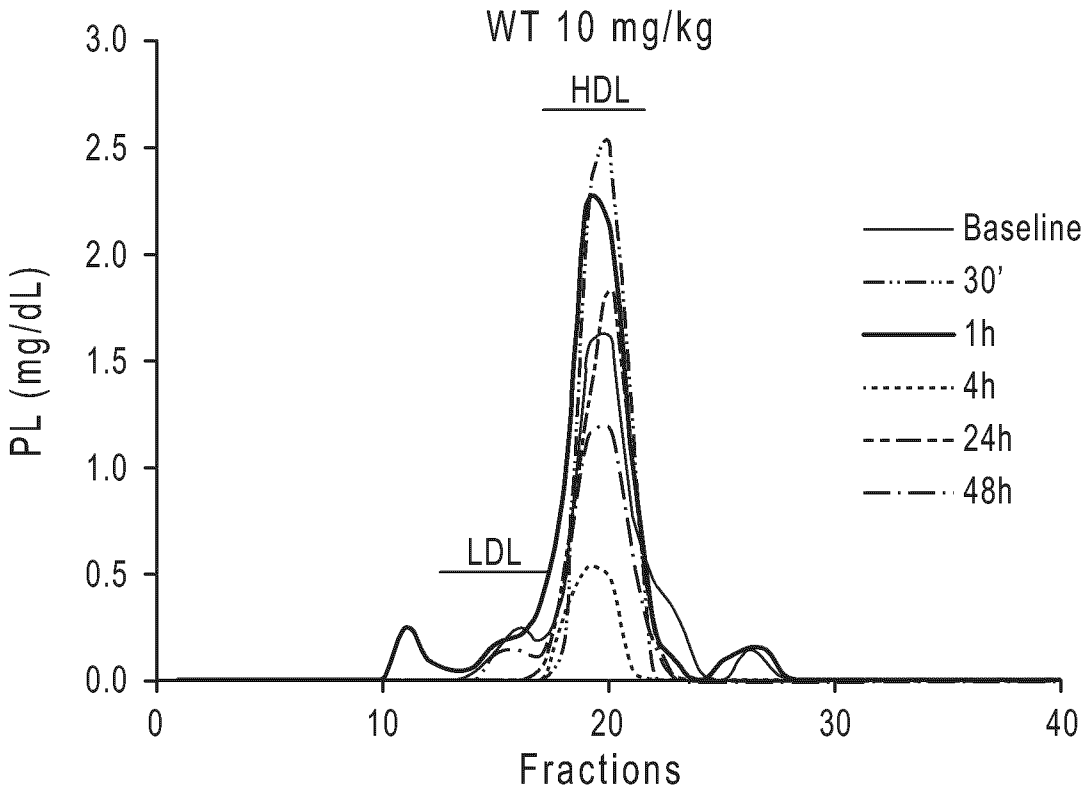

WT 10 mg/kg
HDL
LDL
PL (mg/dL)
Fractions
Baseline
30'
1h
4h
24h
48h
3.0
2.5
2.0
1.5
1.0
0.5
0.0
0
10
20
30
40

Fig. 4A

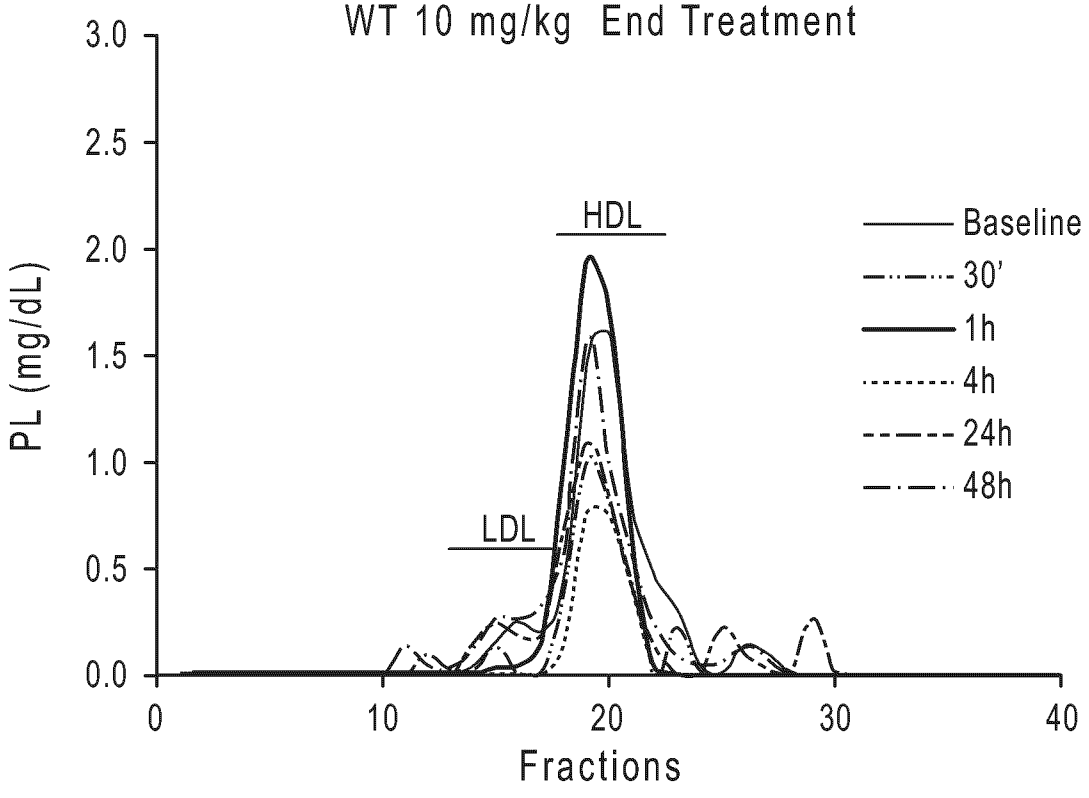

WT 10 mg/kg End Treatment
HDL
LDL
PL (mg/dL)
Fractions
Baseline
30'
1h
4h
24h
48h
3.0
2.5
2.0
1.5
1.0
0.5
0.0
0
10
20
30
40

Fig. 4B

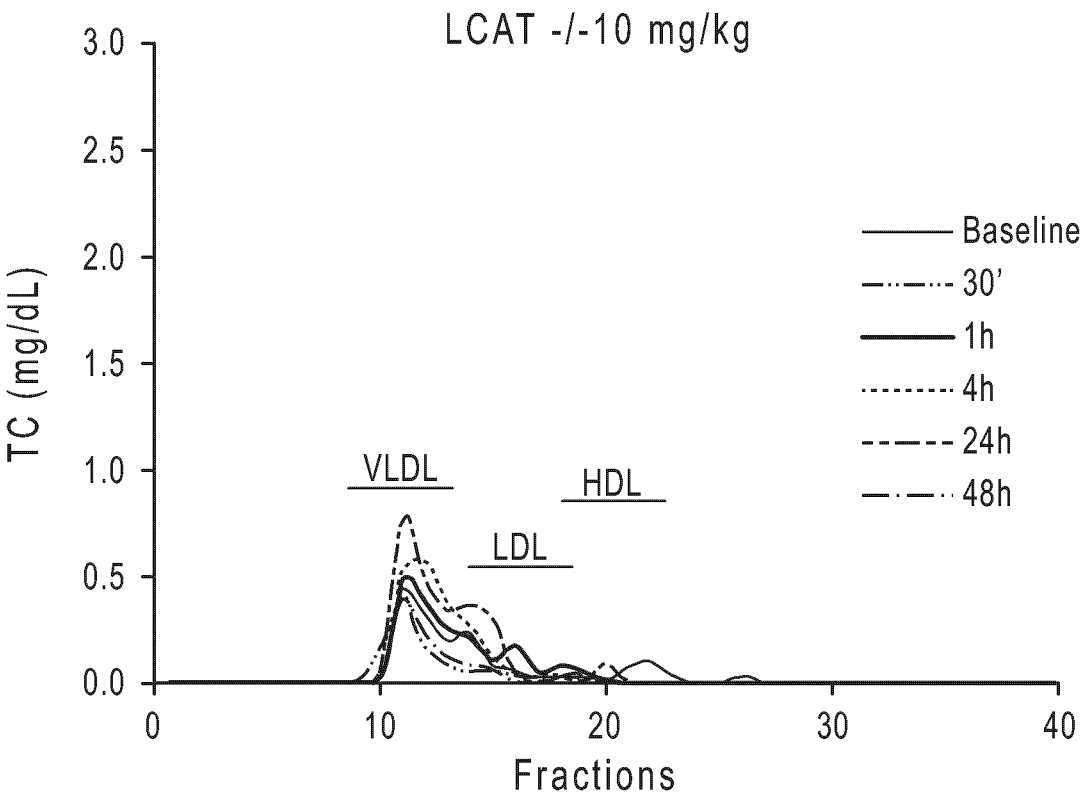

LCAT -/-10 mg/kg
TC (mg/dL)
3.0
2.5
2.0
1.5
1.0
0.5
0.0
0
10
20
30
40
Fractions
VLDL
LDL
HDL
Baseline
30'
1h
4h
24h
48h

Fig. 5A

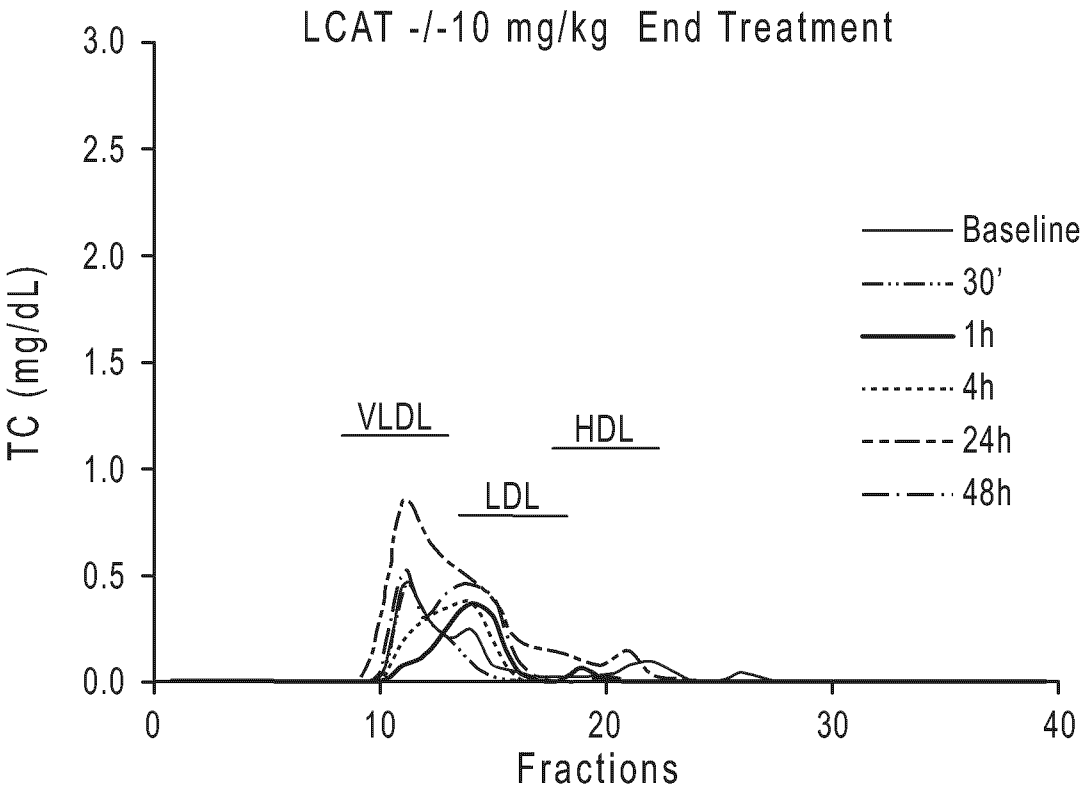

LCAT -/-10 mg/kg End Treatment
TC (mg/dL)
3.0
2.5
2.0
1.5
1.0
0.5
0.0
0
10
20
30
40
Fractions
VLDL
LDL
HDL
Baseline
30'
1h
4h
24h
48h

Fig. 5B

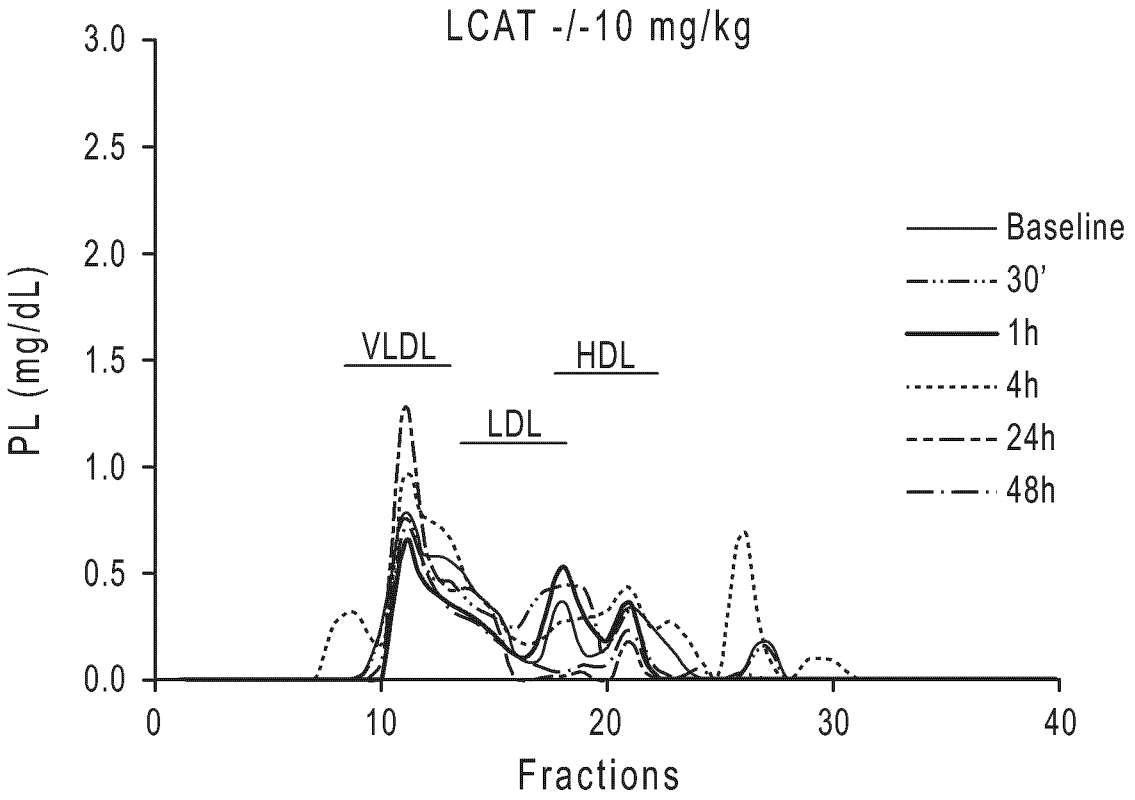

LCAT -/-10 mg/kg
PL (mg/dL)
3.0
2.5
2.0
1.5
1.0
0.5
0.0
VLDL
HDL
LDL
Baseline
30'
1h
4h
24h
48h
0
10
20
30
40
Fractions

Fig. 6A

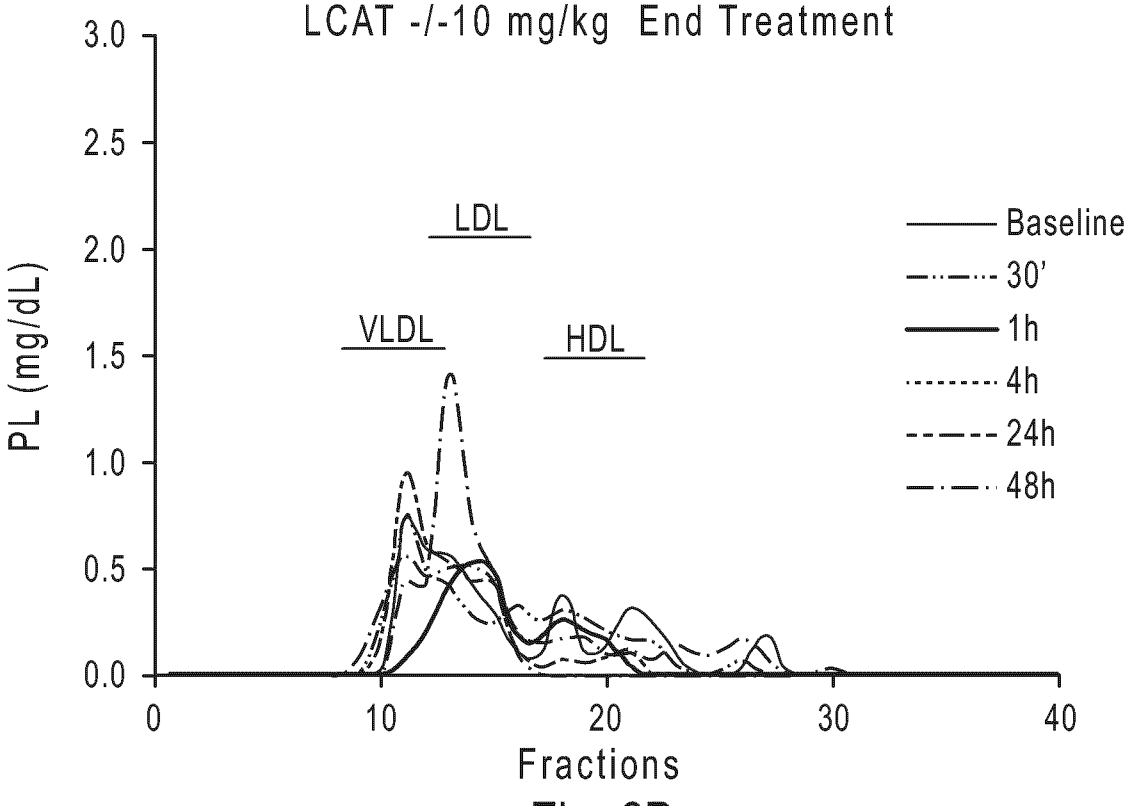

LCAT -/-10 mg/kg End Treatment
PL (mg/dL)
3.0
2.5
2.0
1.5
1.0
0.5
0.0
LDL
VLDL
HDL
Baseline
30'
1h
4h
24h
48h
0
10
20
30
40
Fractions

Fig. 6B

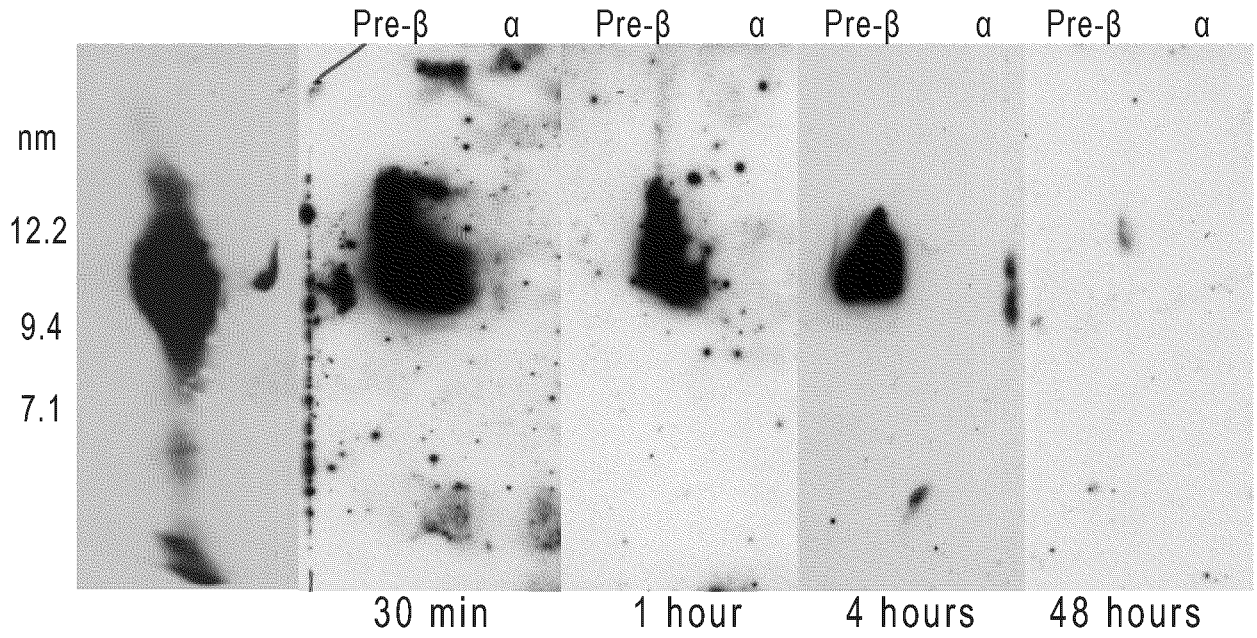

Pre-β
α
Pre-β
α
Pre-β
α
Pre-β
α
nm
12.2
9.4
7.1
30 min
1 hour
4 hours
48 hours

Fig. 7A

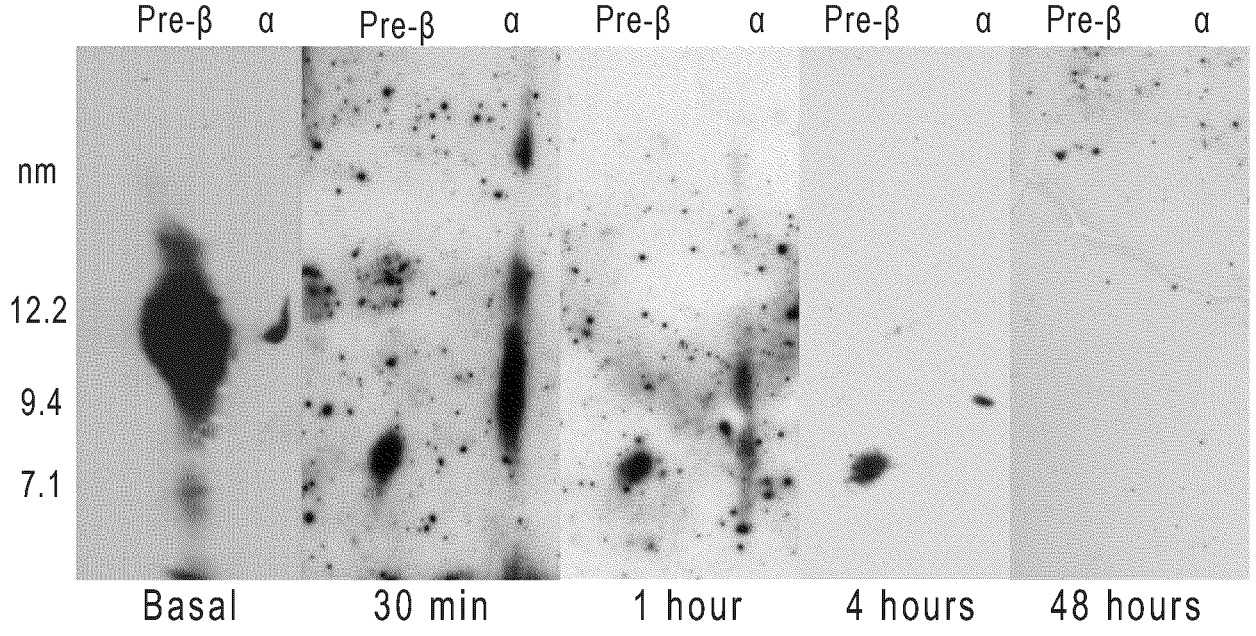

Pre-β
α
Pre-β
α
Pre-β
α
Pre-β
α
Pre-β
α
nm
12.2
9.4
7.1
Basal
30 min
1 hour
4 hours
48 hours

Fig. 7B

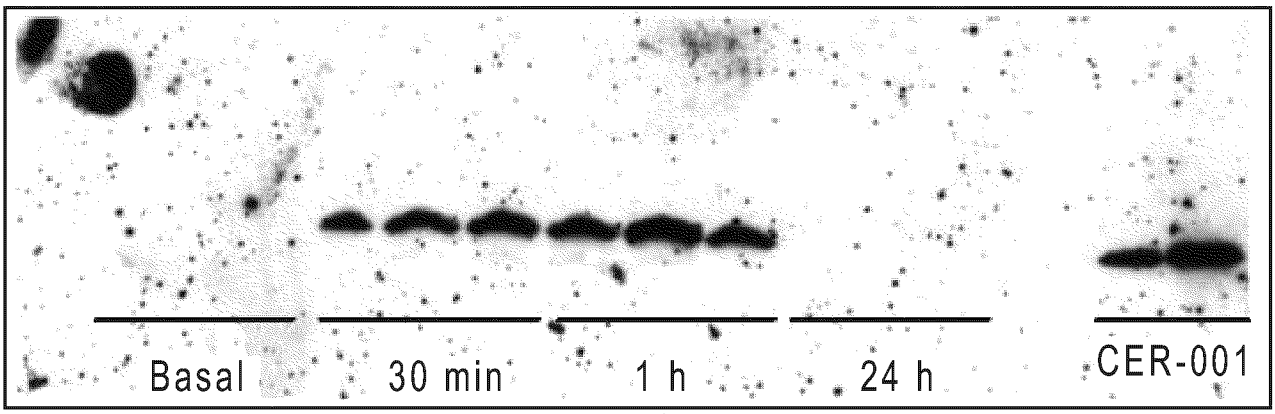


Fig. 8A

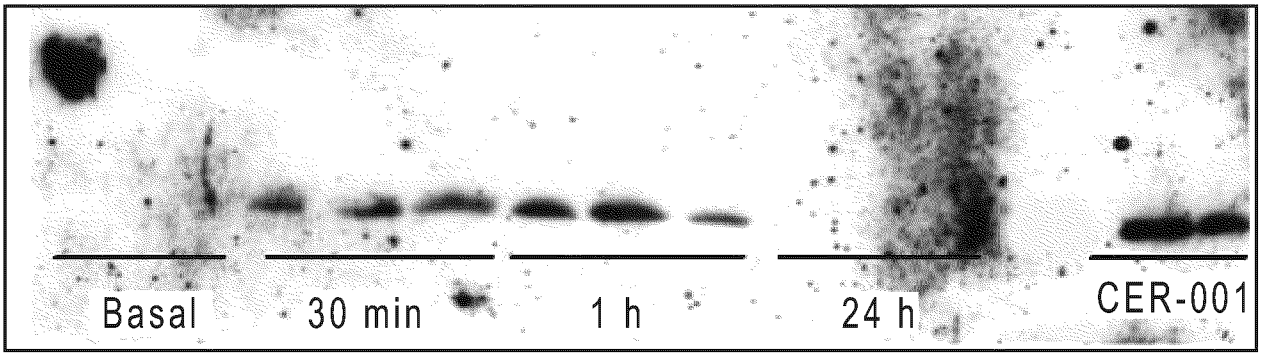


Fig. 8B

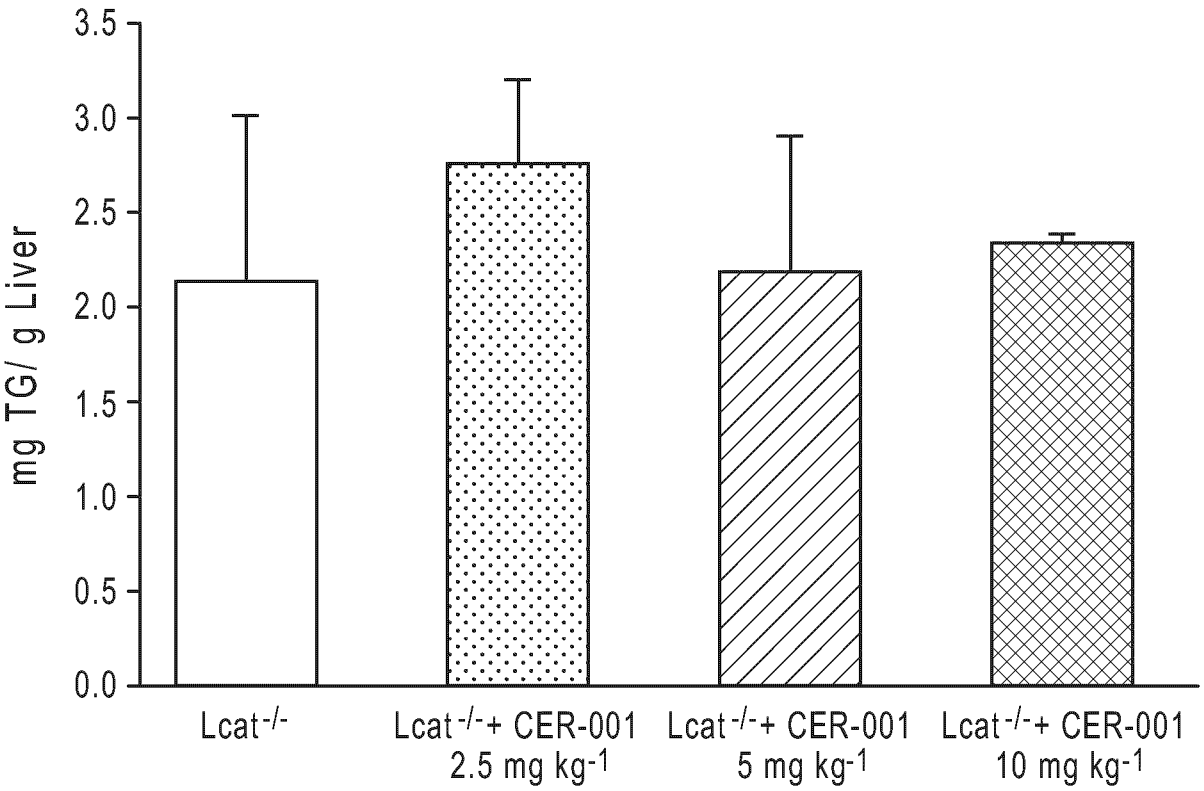

3.5
3.0
2.5
2.0
1.5
1.0
0.5
0.0
mg TG/ g Liver
Lcat-/-
Lcat-/-+ CER-001 2.5 mg kg-1
Lcat-/-+ CER-001 5 mg kg-1
Lcat-/-+ CER-001 10 mg kg-1

Fig. 9

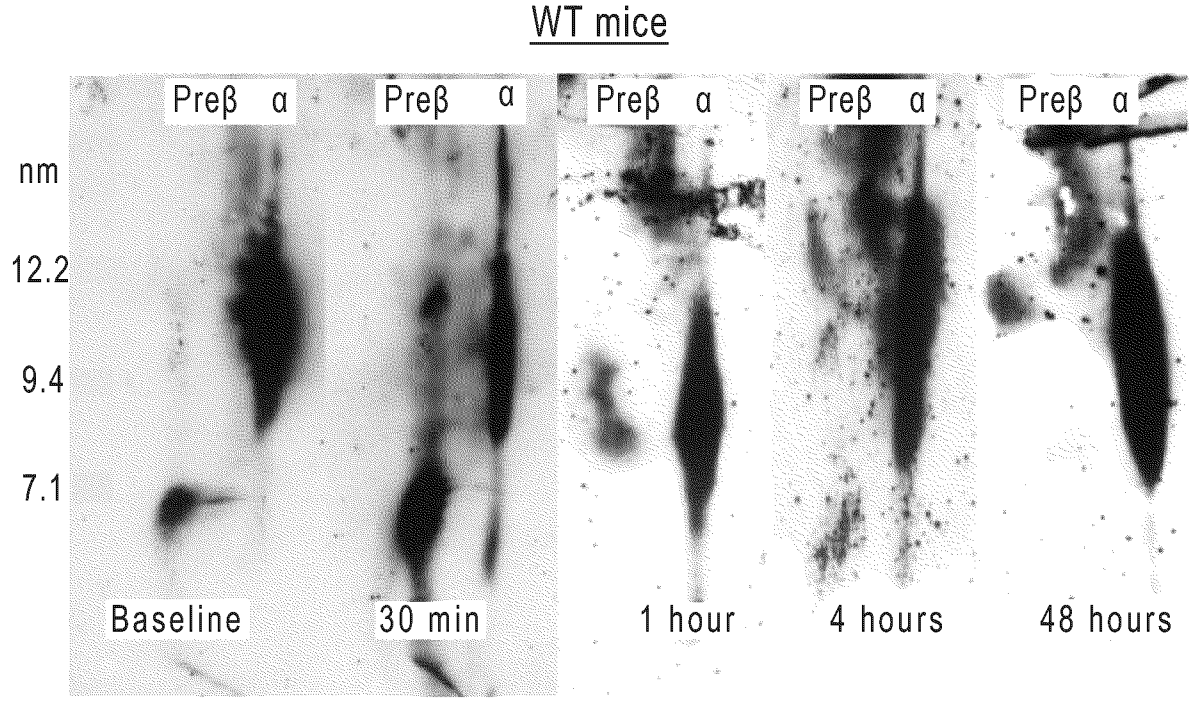


Fig. 10A

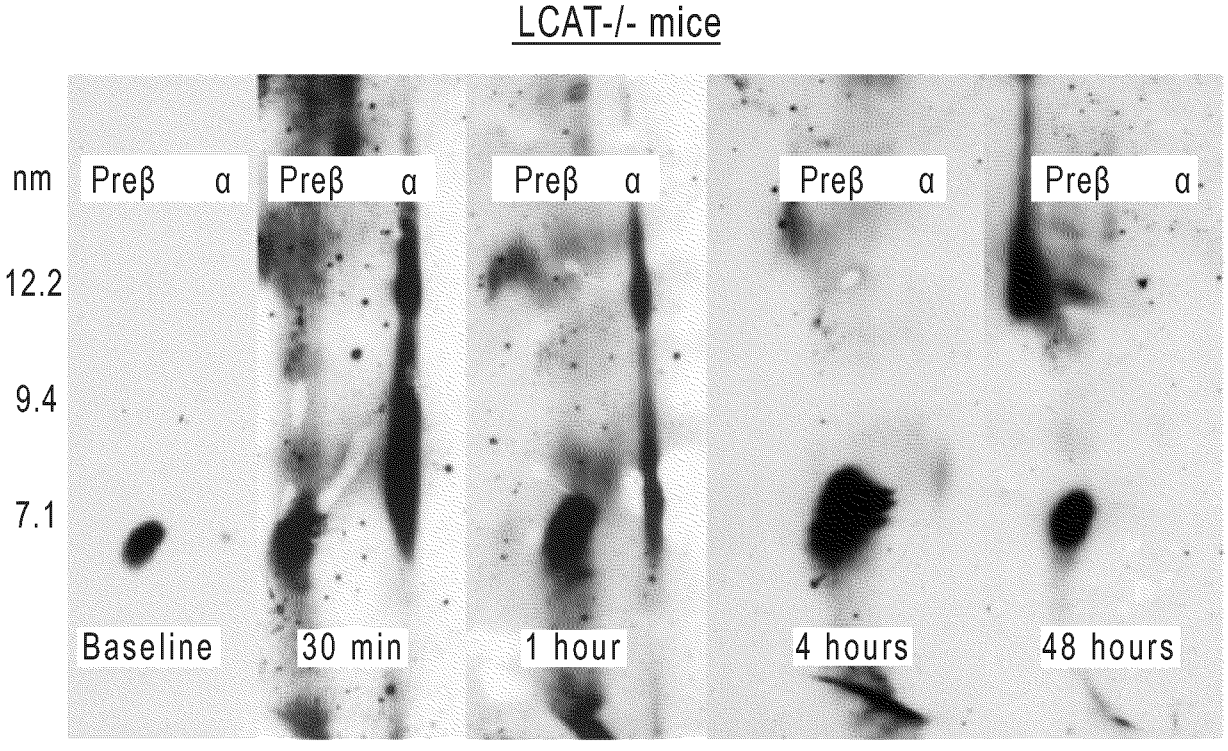


Fig. 10B

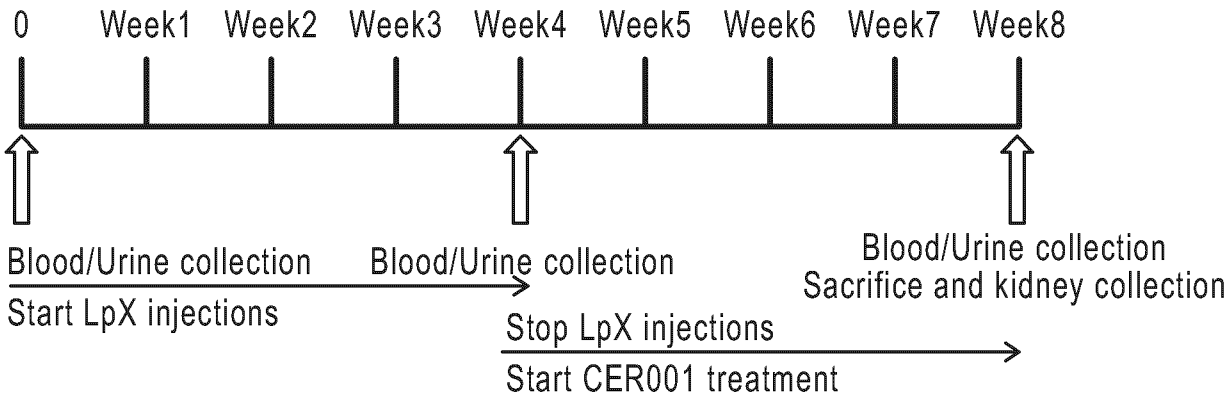

0
Week1
Week2
Week3
Week4
Week5
Week6
Week7
Week8
Blood/Urine collection
Start LpX injections
Blood/Urine collection
Stop LpX injections
Start CER001 treatment
Blood/Urine collection
Sacrifice and kidney collection

Fig. 11

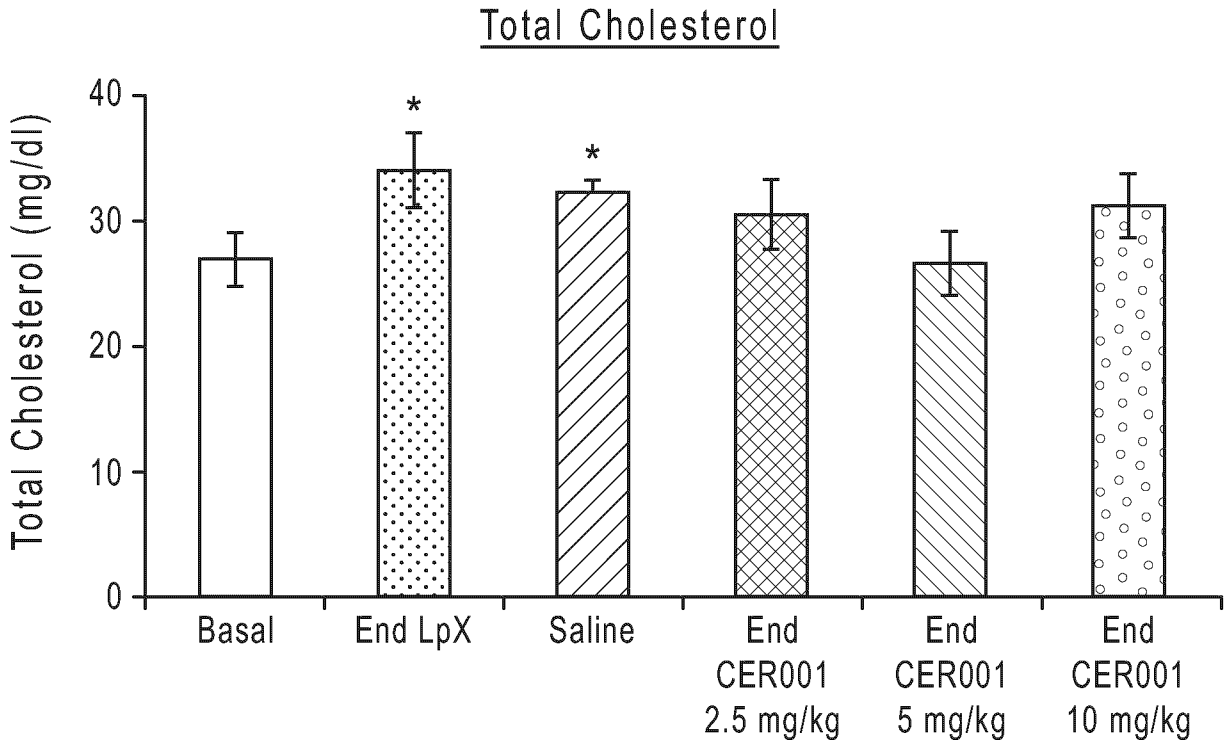

Total Cholesterol
Total Cholesterol (mg/dl)
40
30
20
10
0
*
*
Basal
End LpX
Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 12

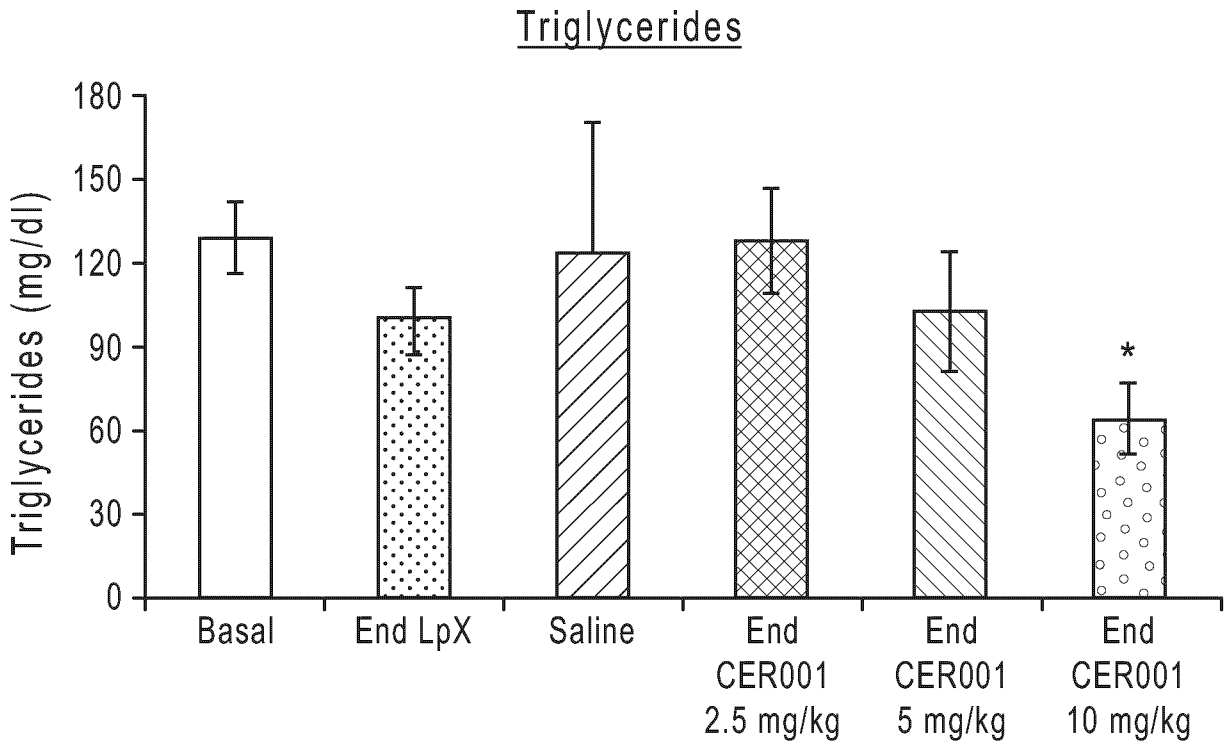

Triglycerides
Triglycerides (mg/dl)
180
150
120
90
60
30
0
*
Basal
End LpX
Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 13

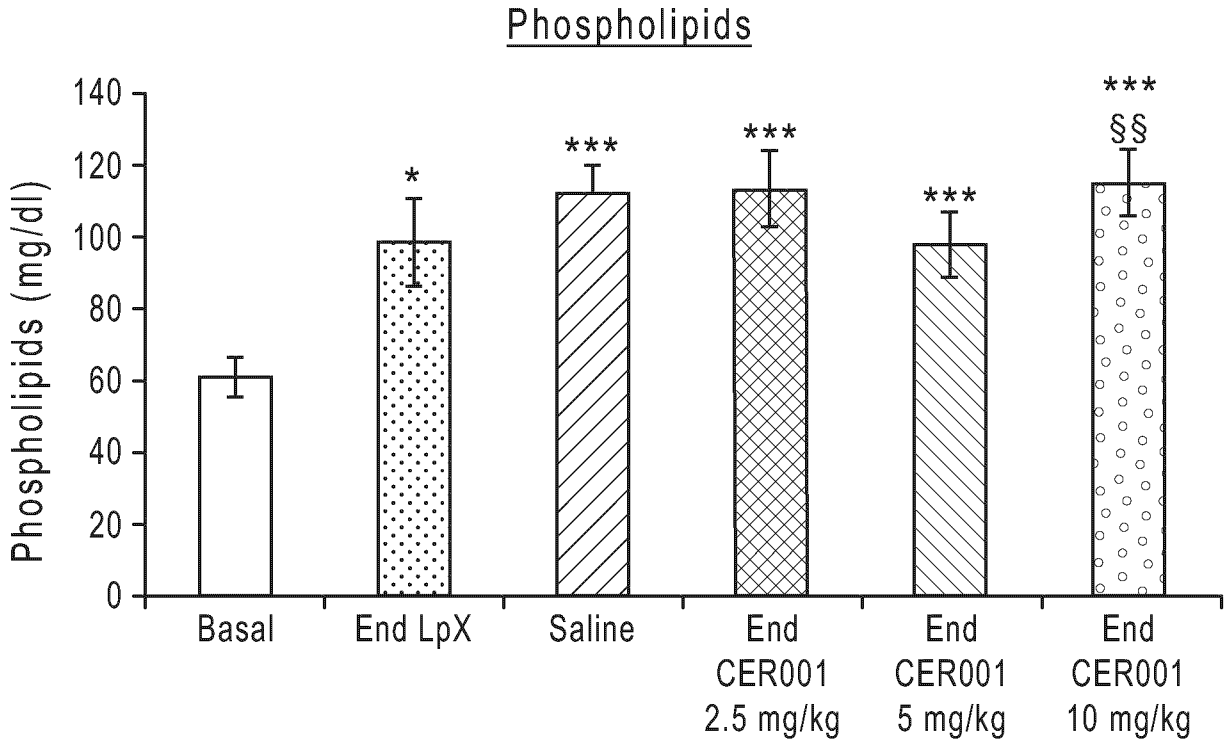

Phospholipids
Phospholipids (mg/dl)
140
120
100
80
60
40
20
0
*
***
***
***
***
§§
Basal
End LpX
Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 14

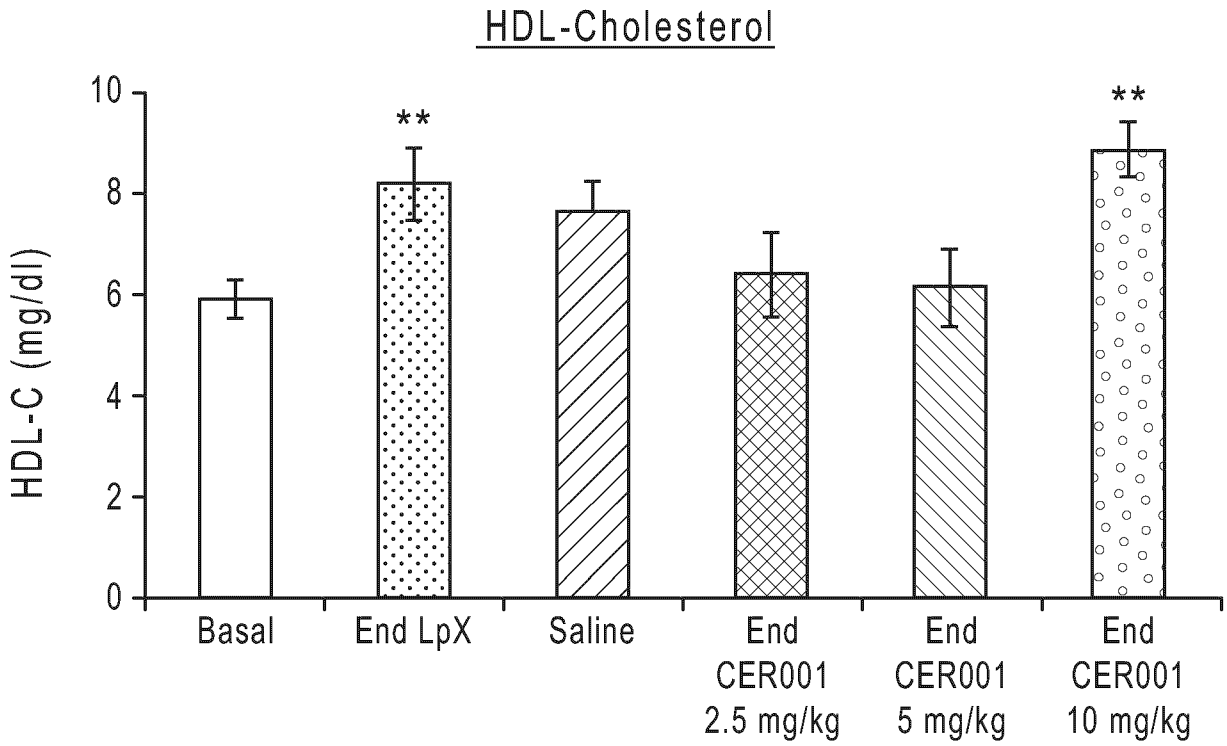

HDL-Cholesterol
HDL-C (mg/dl)
10
8
6
4
2
0
**
**
Basal
End LpX
Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 15

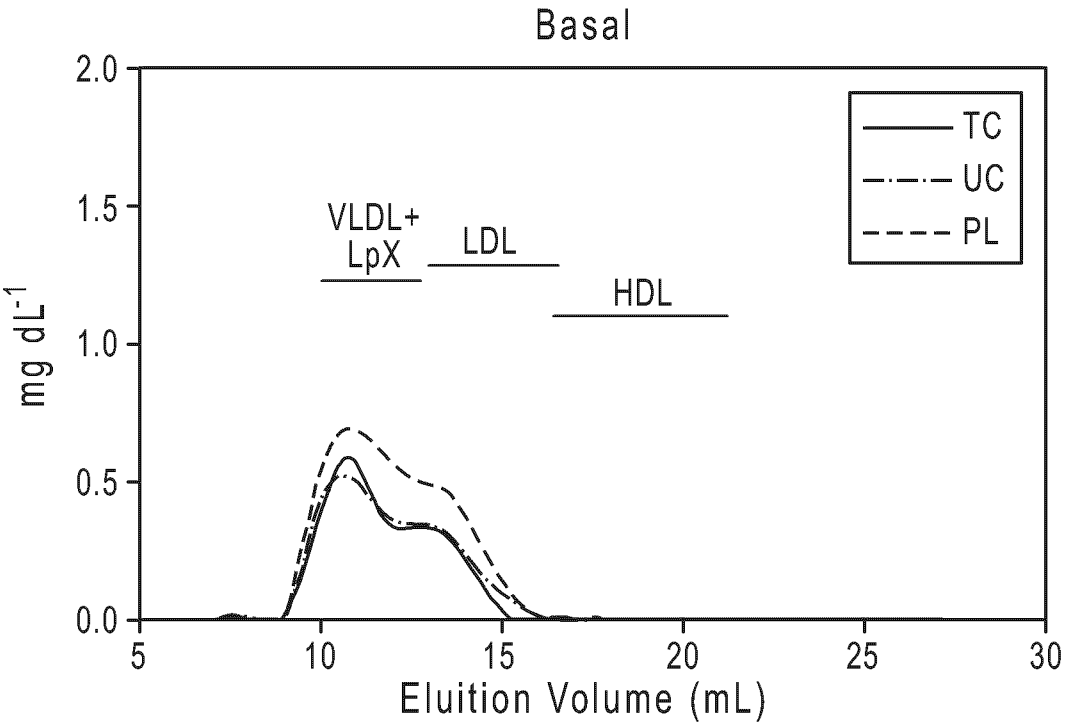


Fig. 16A

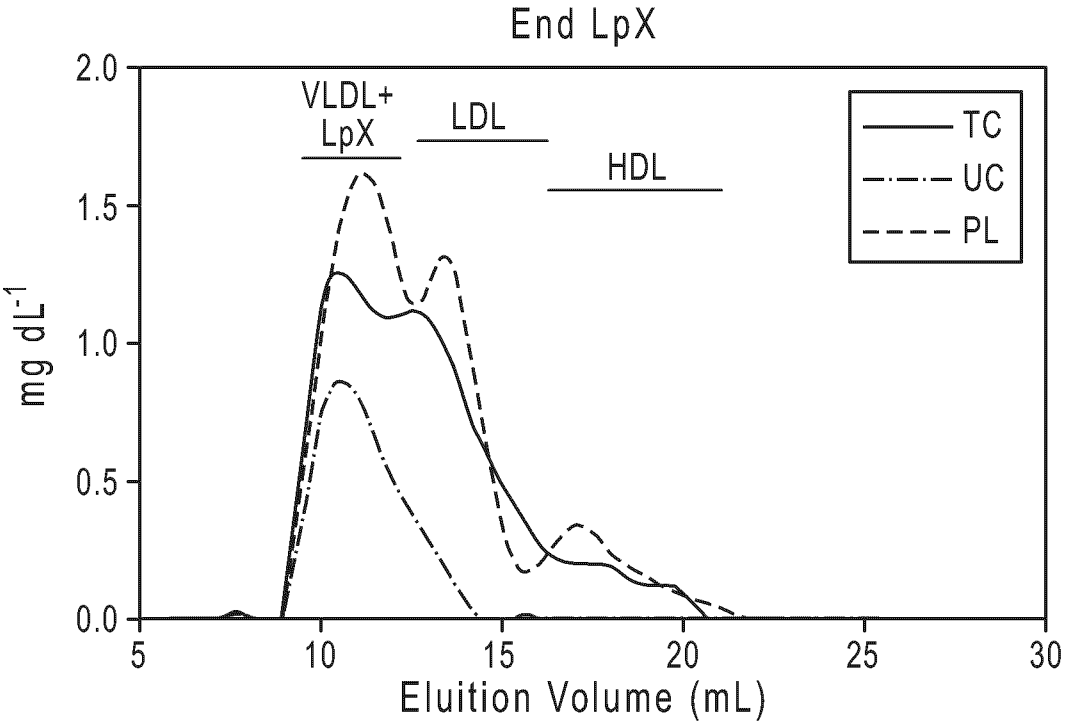


Fig. 16B

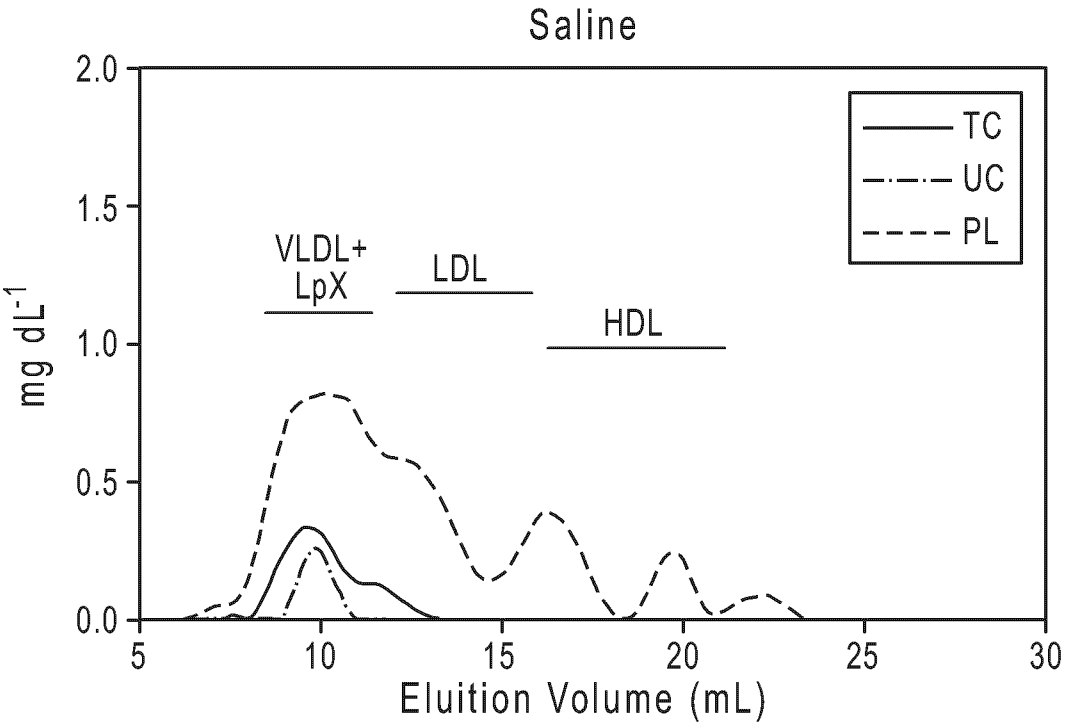


Fig. 16C

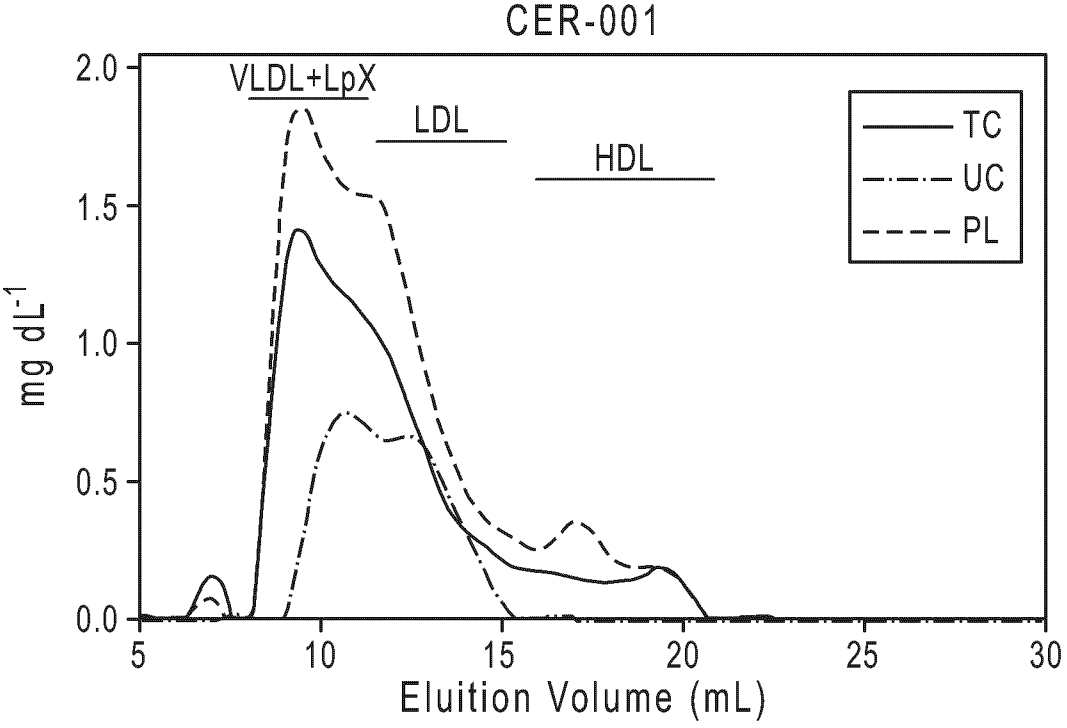


Fig. 16D

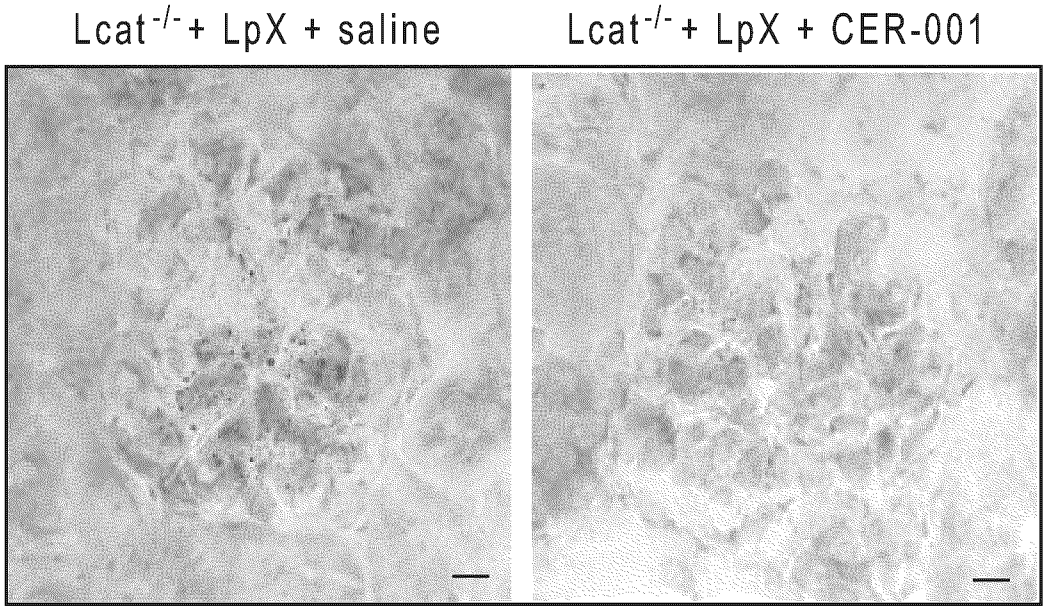


Fig. 17A

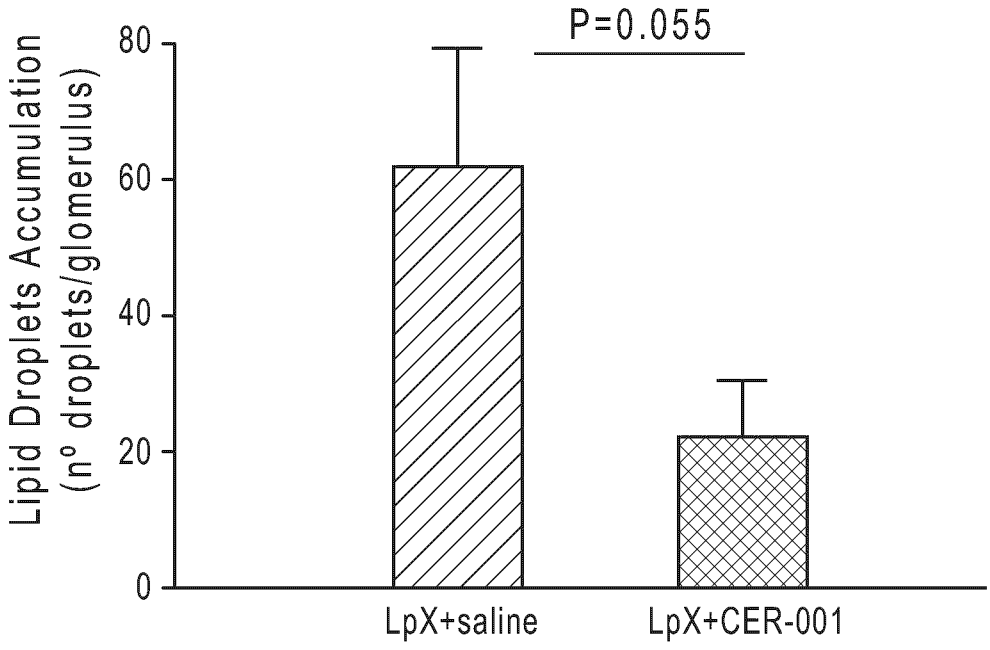


Fig. 17B

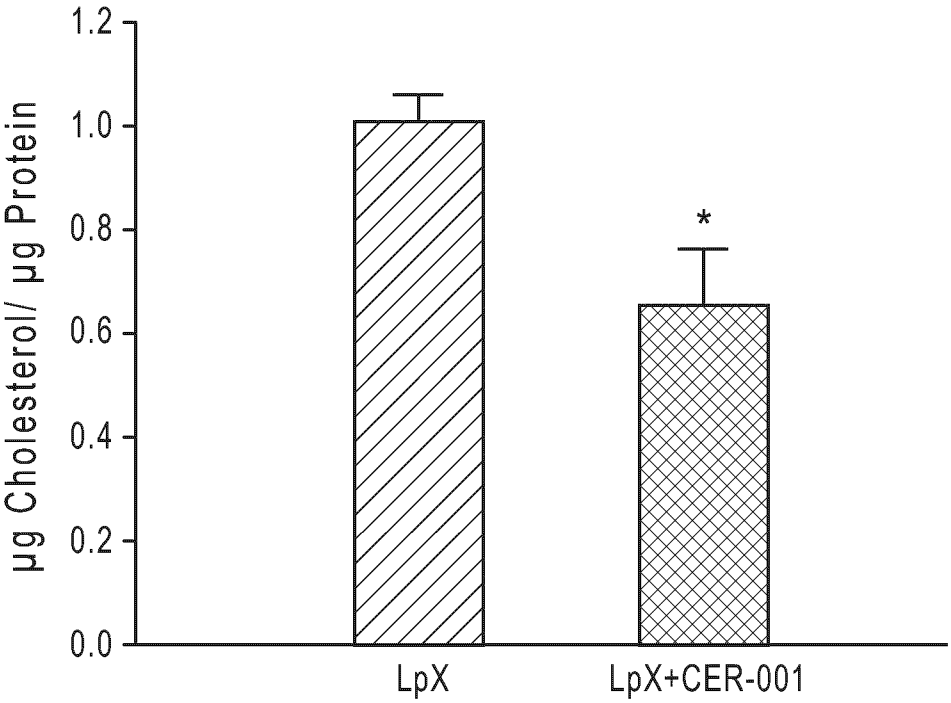

1.2
1.0
0.8
0.6
0.4
0.2
0.0
µg Cholesterol/ µg Protein
*
LpX
LpX+CER-001

Fig. 18

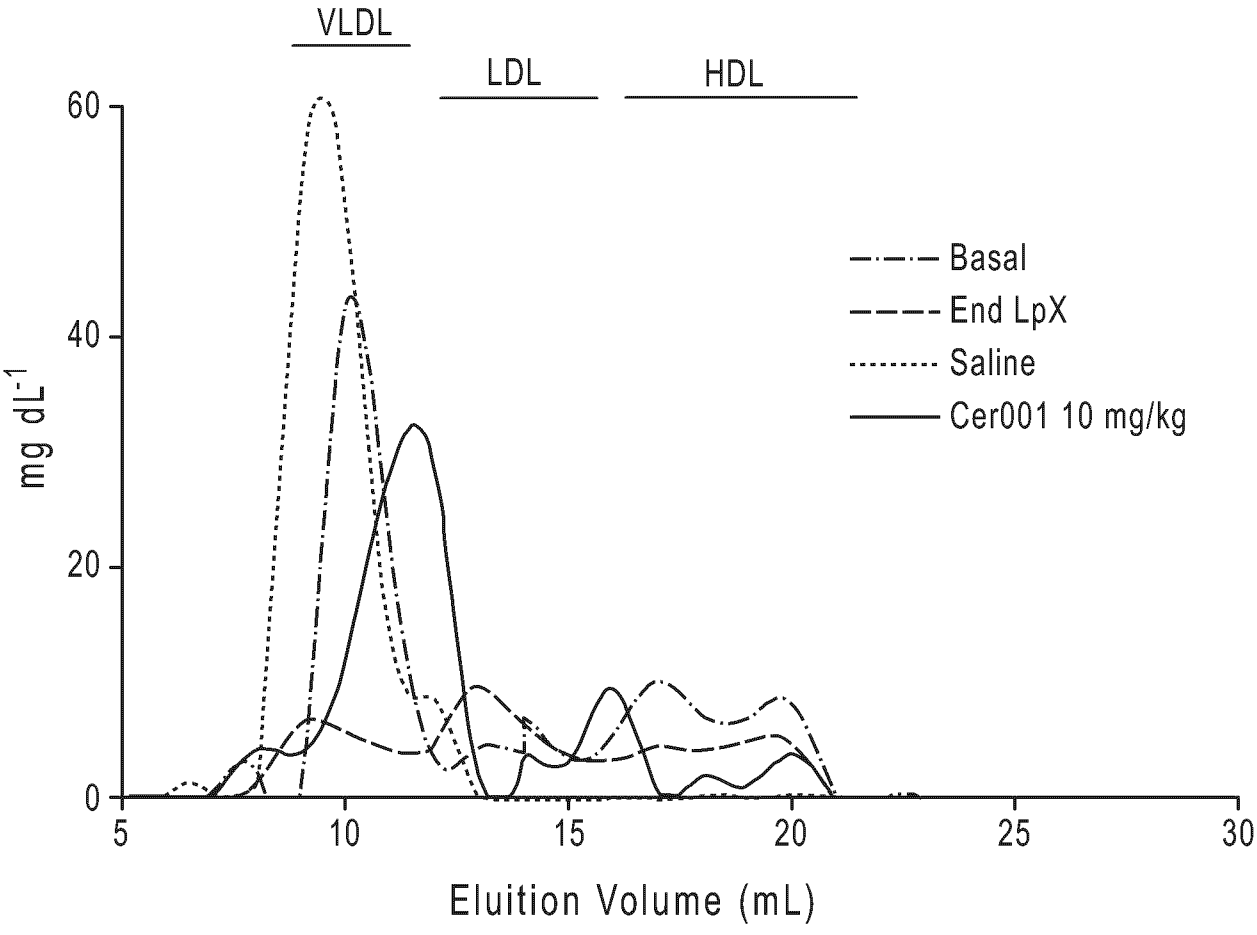

VLDL
LDL
HDL
60
40
20
0
mg dL-1
5
10
15
20
25
30
Eluition Volume (mL)
Basal
End LpX
Saline
Cer001 10 mg/kg

Fig. 19

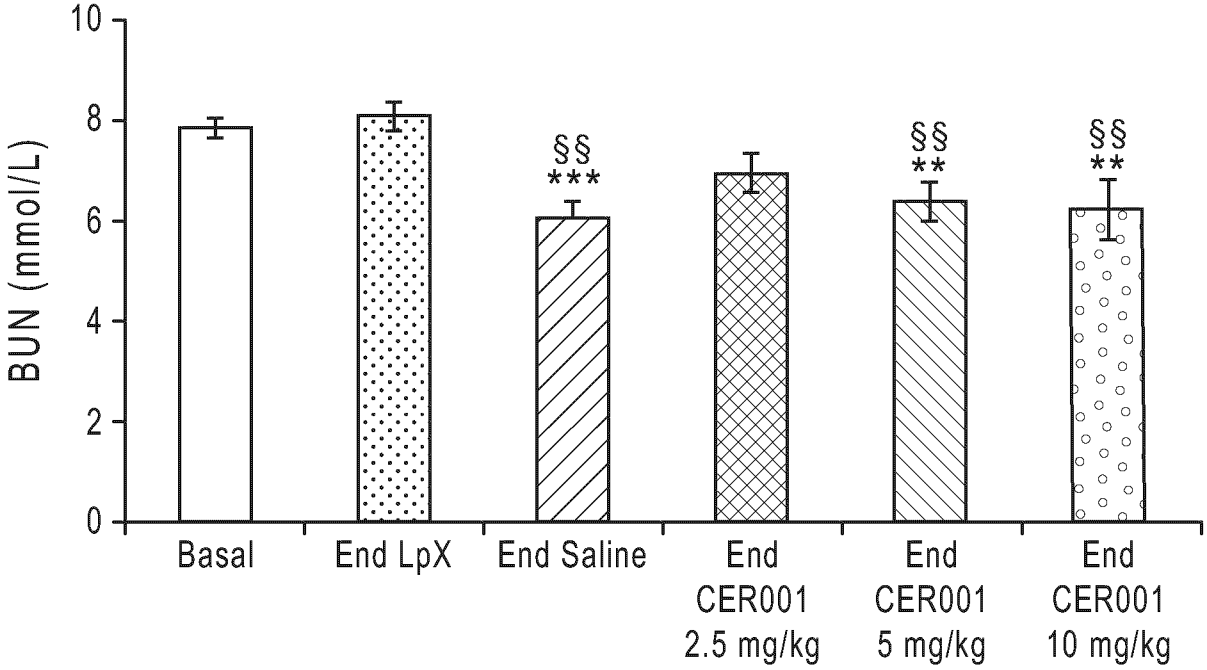

BUN (mmol/L)
10
8
6
4
2
0
§§
***
§§
**
§§
**
Basal
End LpX
End Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 20

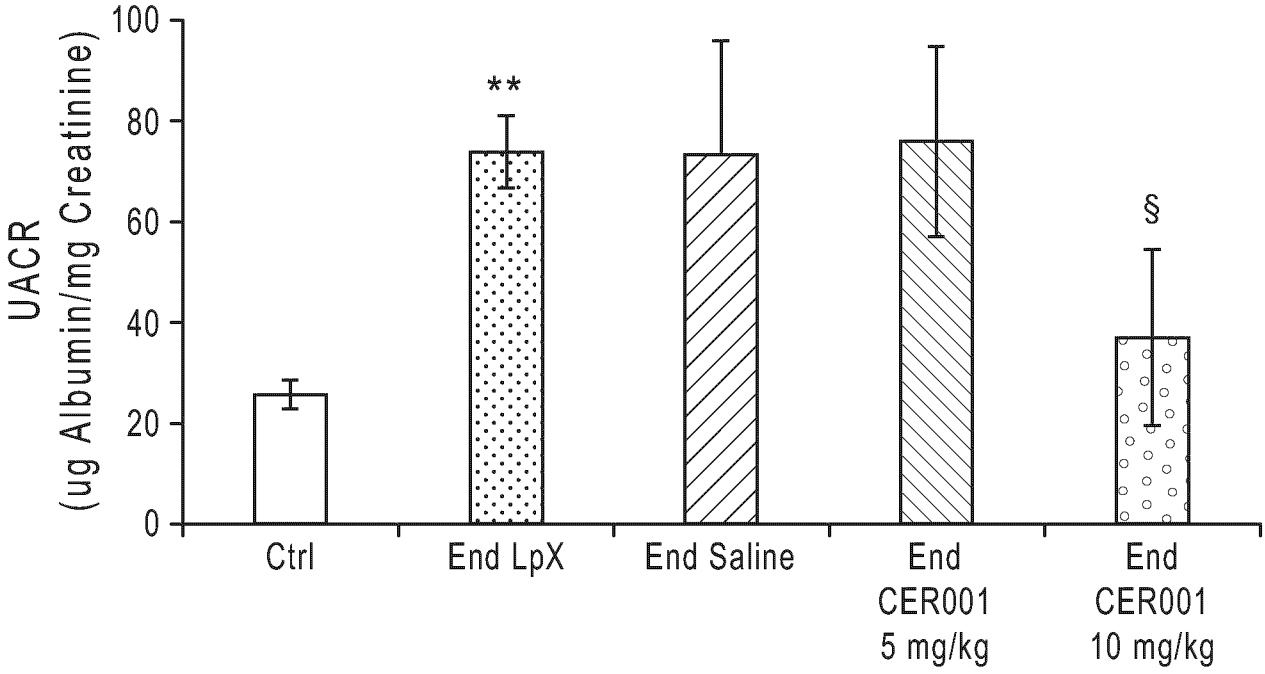

UACR (ug Albumin/mg Creatinine)
100
80
60
40
20
0
**
§
Ctrl
End LpX
End Saline
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 21

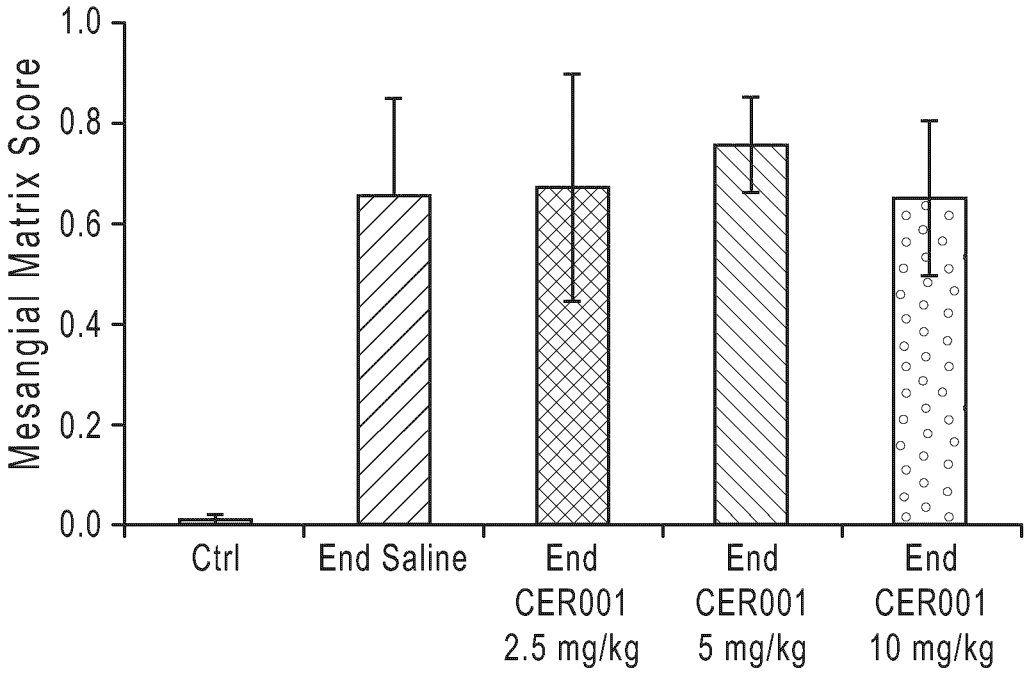

Mesangial Matrix Score
1.0
0.8
0.6
0.4
0.2
0.0
Ctrl
End Saline
End CER001 2.5 mg/kg
End CER001 5 mg/kg
End CER001 10 mg/kg

Fig. 22

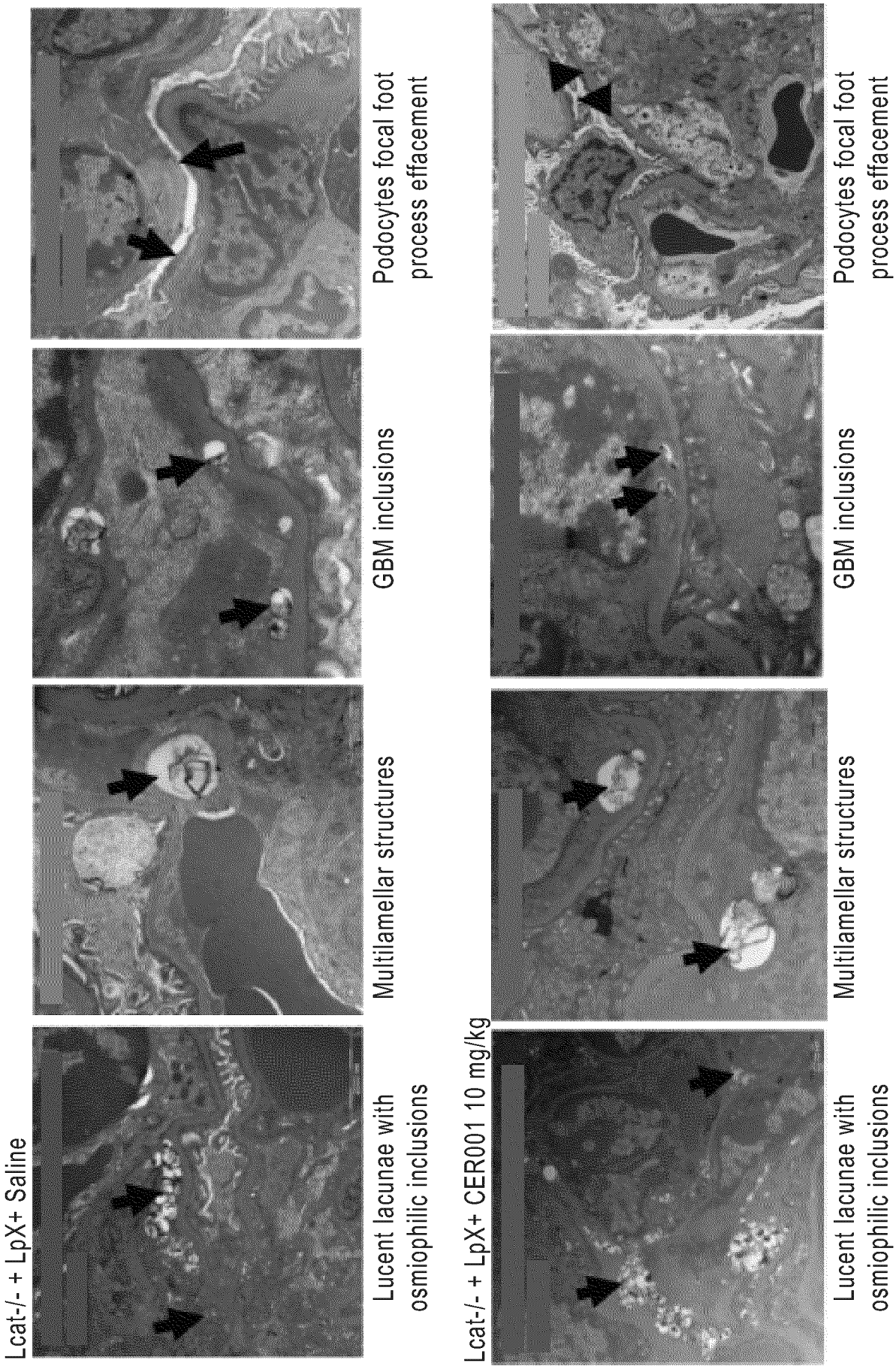

Lcat-/- + LpX+ Saline
Lucent lacunae with osmiophilic inclusions
Multilamellar structures
GBM inclusions
Podocytes focal foot process effacement
Lcat-/- + LpX+ CER001 10 mg/kg
Lucent lacunae with osmiophilic inclusions
Multilamellar structures
GBM inclusions
Podocytes focal foot process effacement

Fig. 23

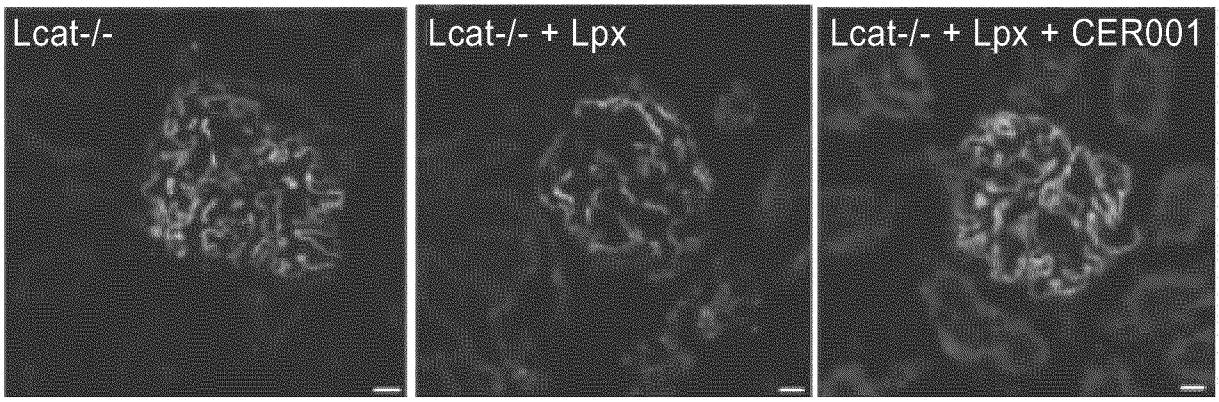


Fig. 24

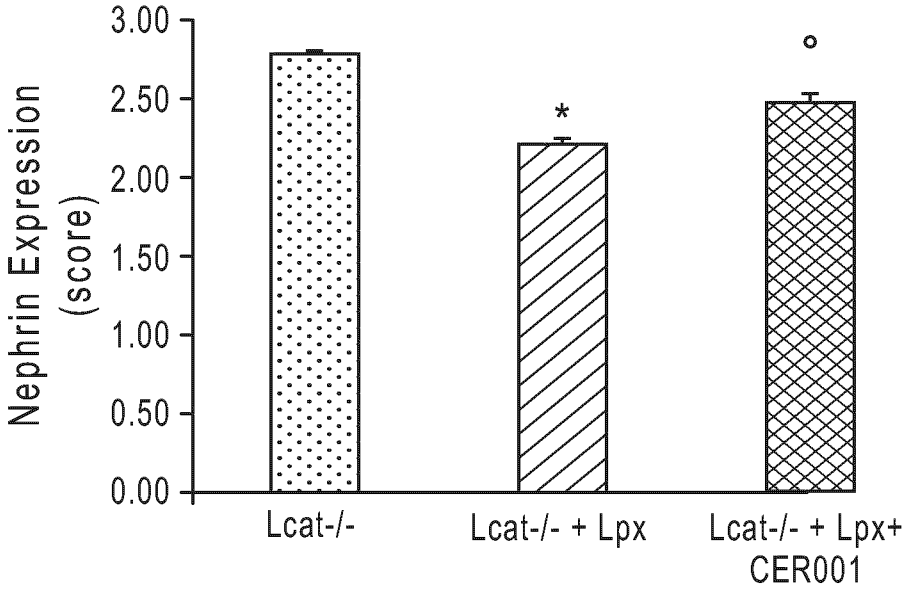


Fig. 25

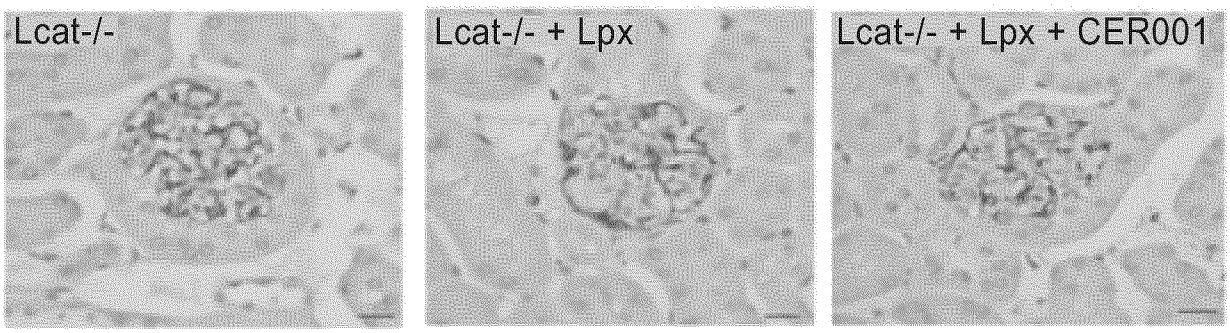


Fig. 26

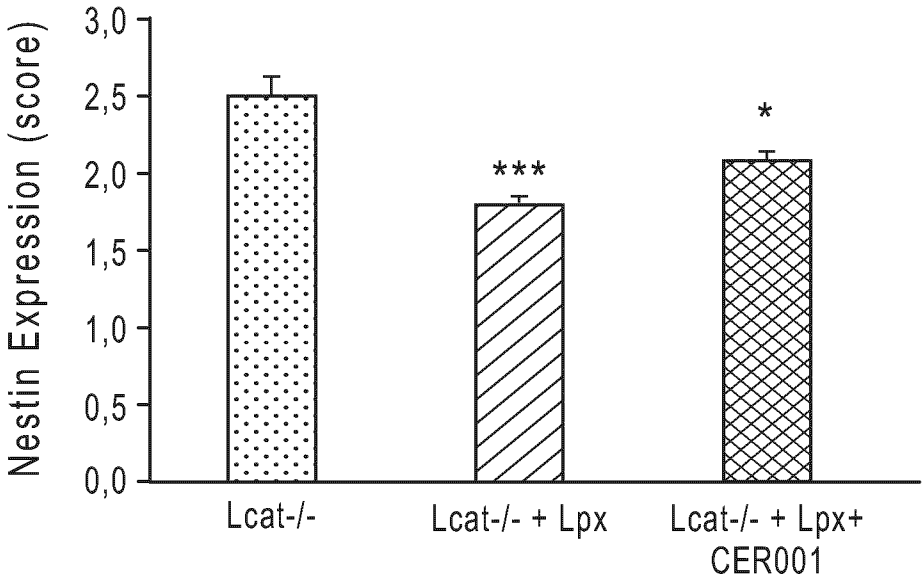


Fig. 27

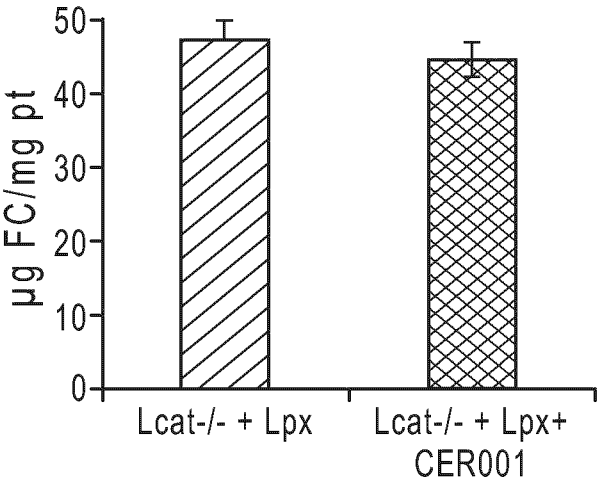


Fig. 28A

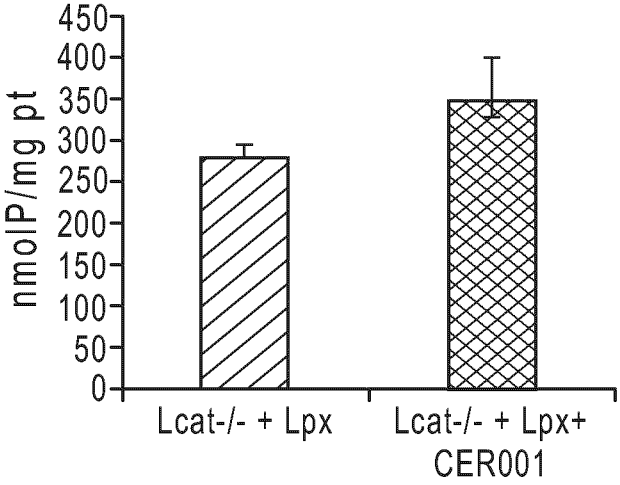


Fig. 28B