

FIG. 1A

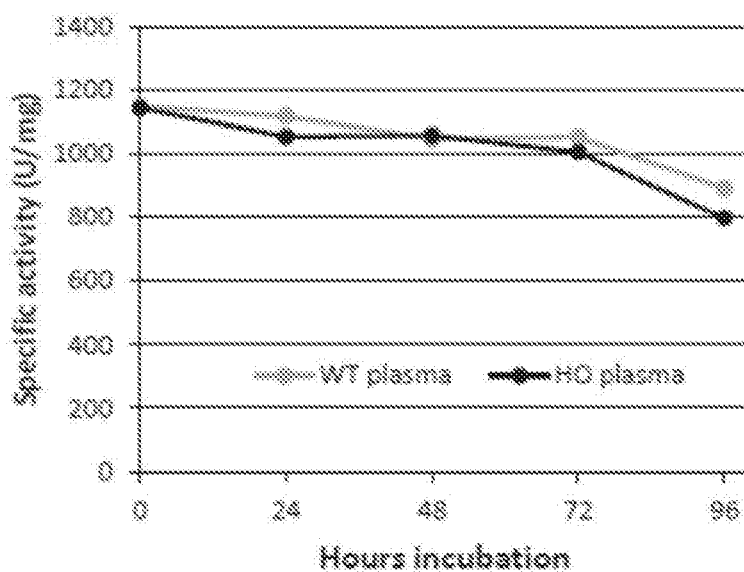


FIG. 1B

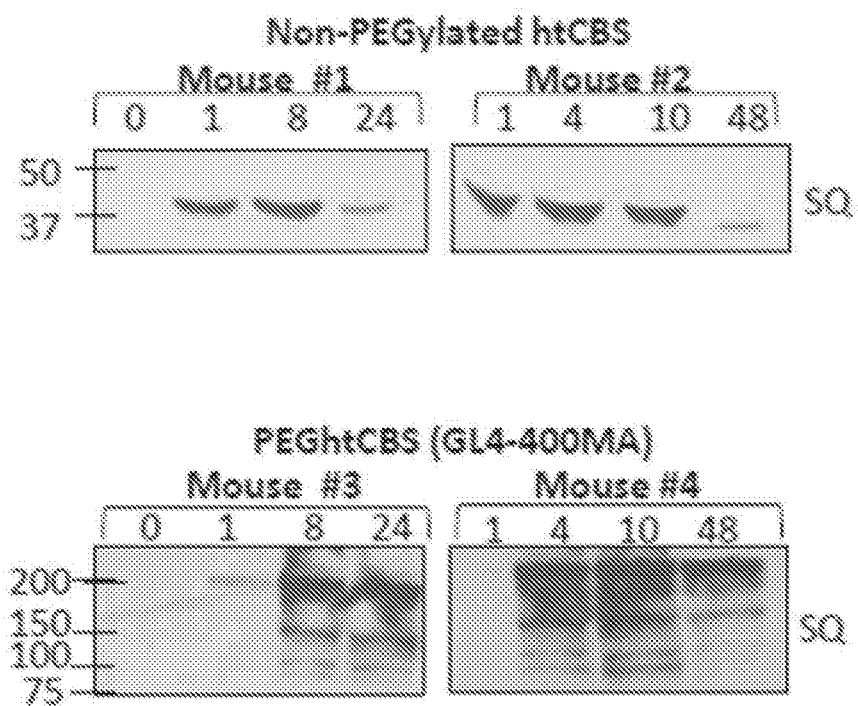


FIG. 2

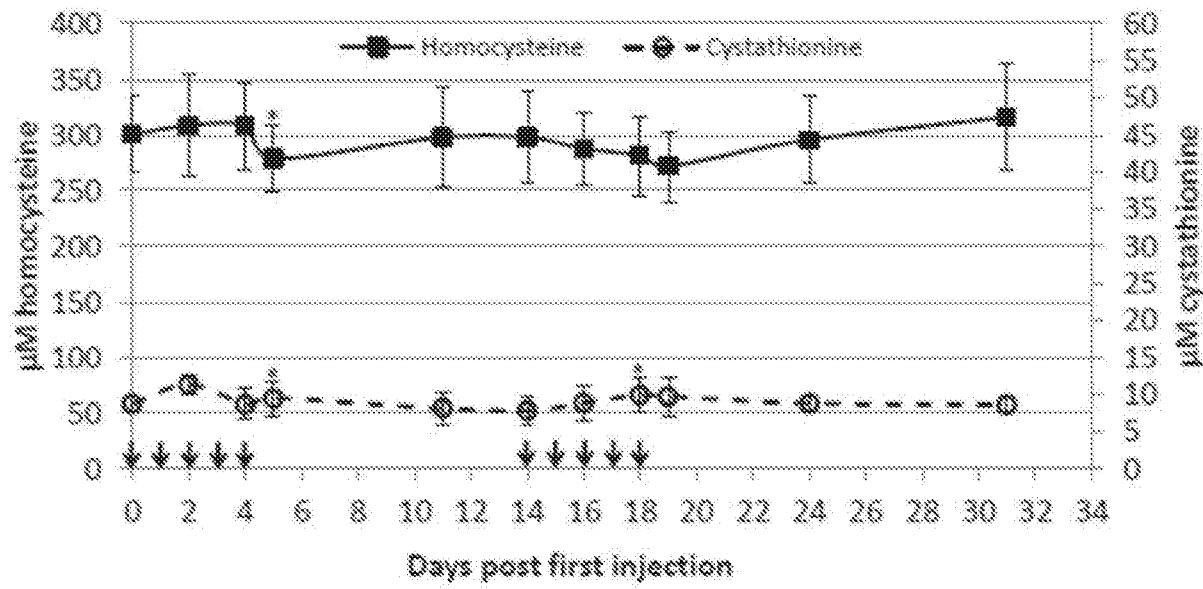


FIG. 3

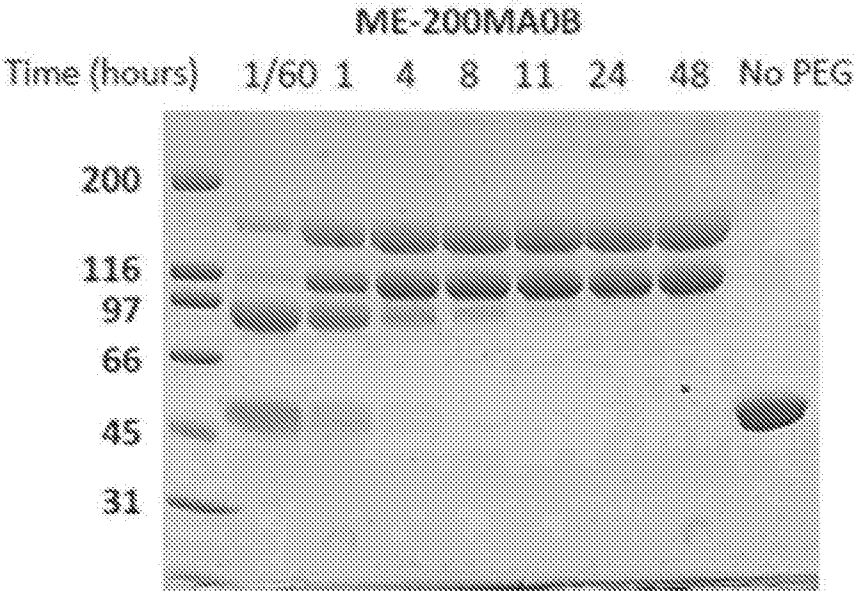


FIG. 4A

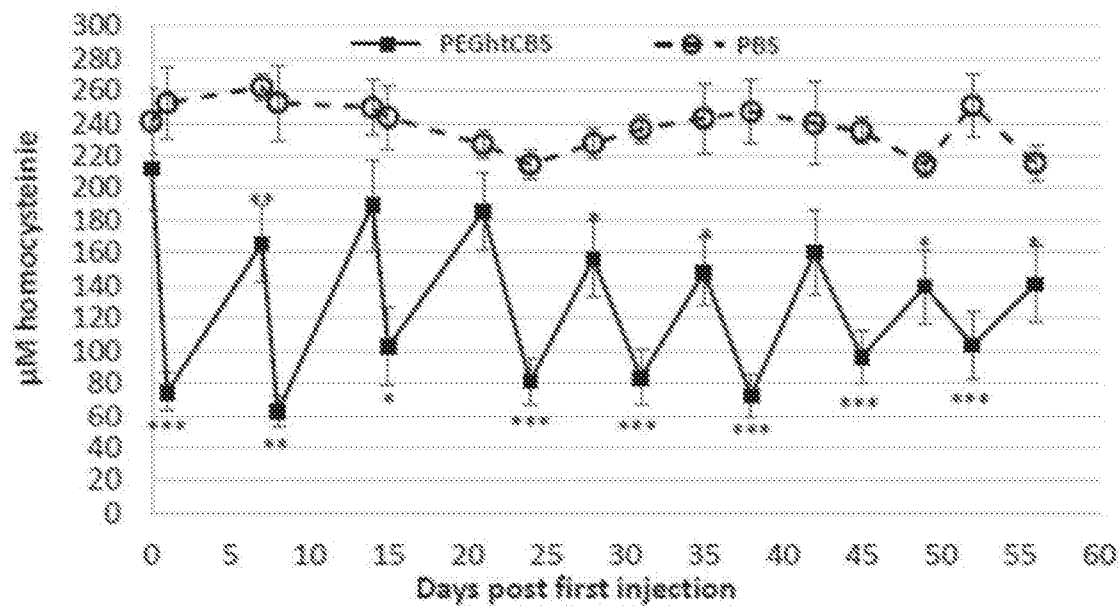


FIG. 4B

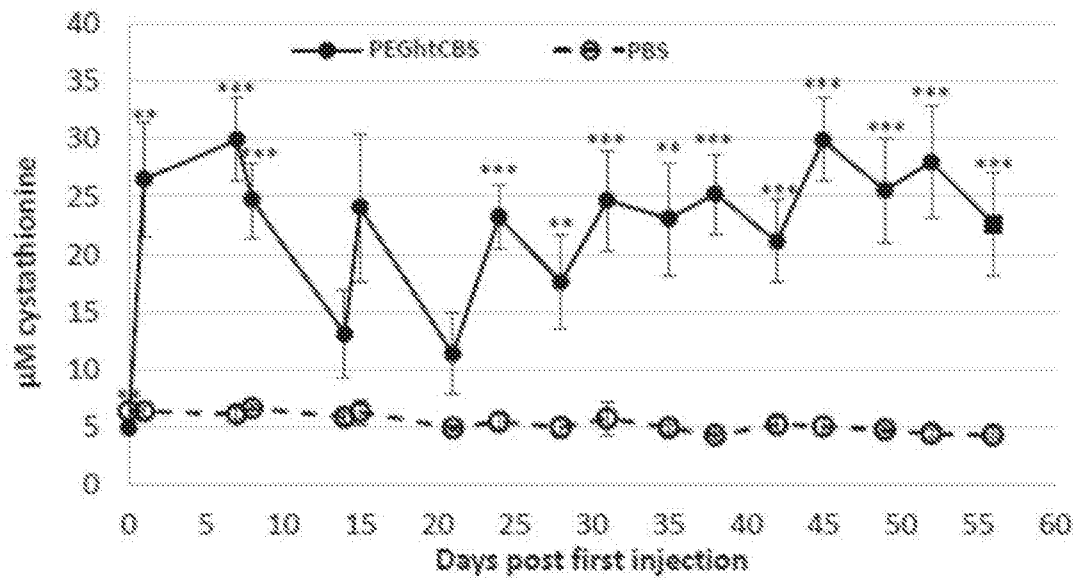


FIG. 4C

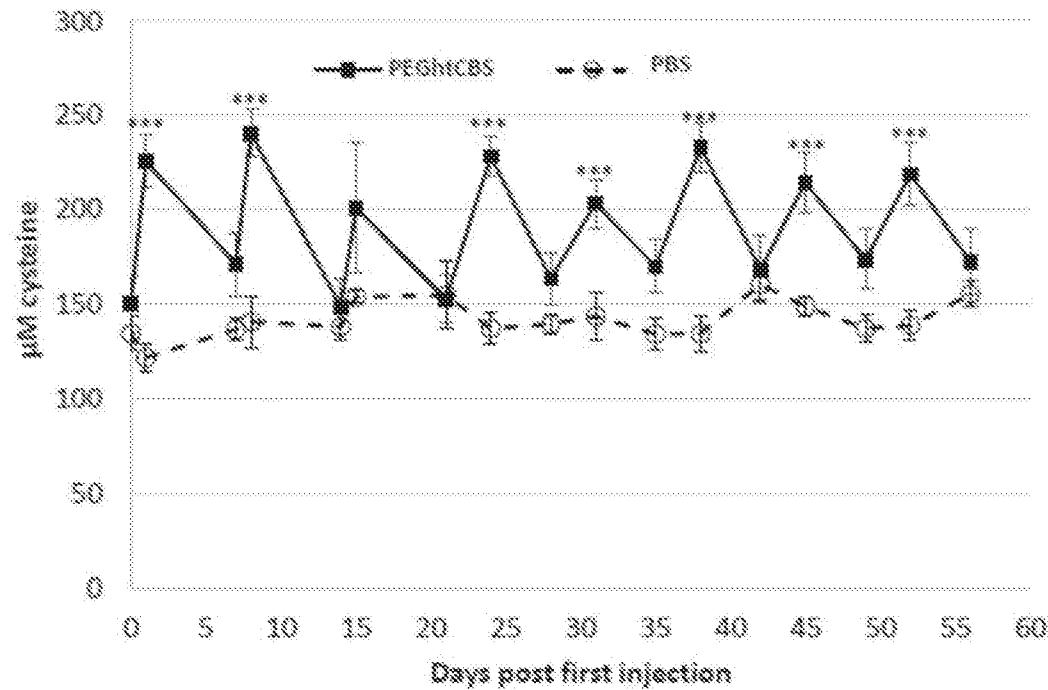


FIG. 4D

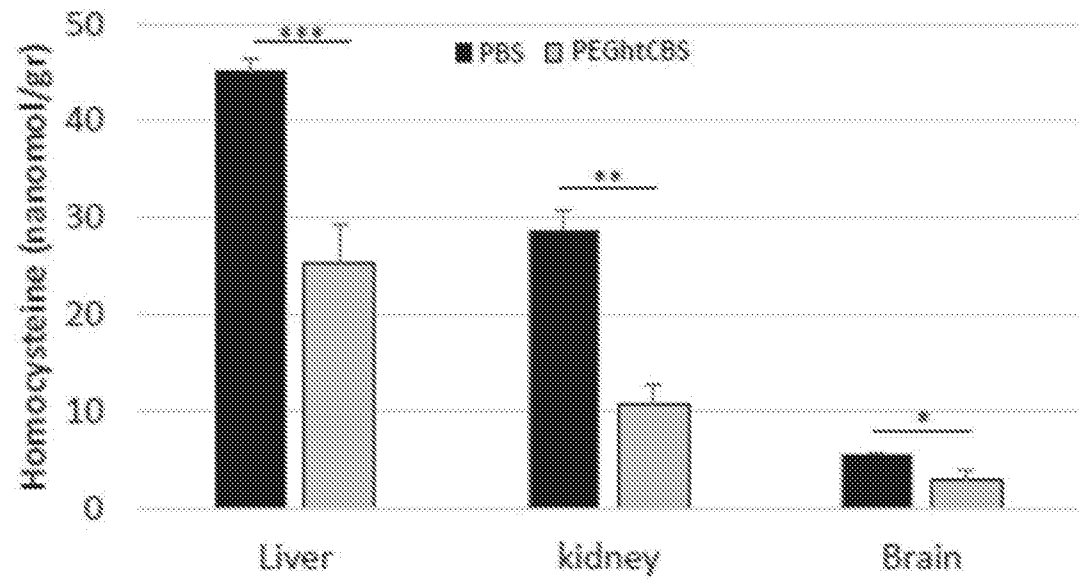


FIG. 5A

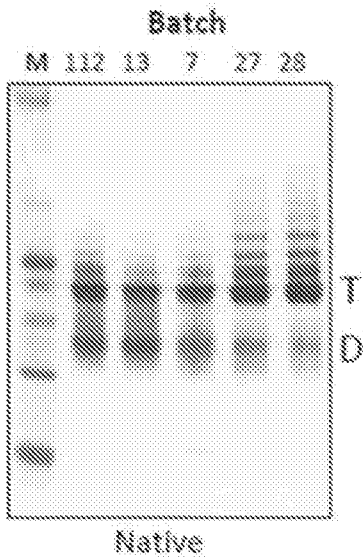


FIG. 5B

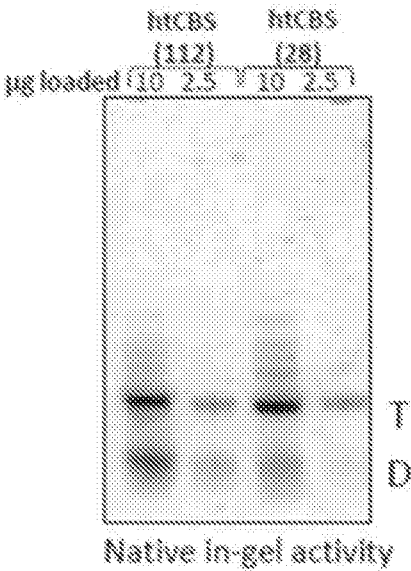


FIG. 5C

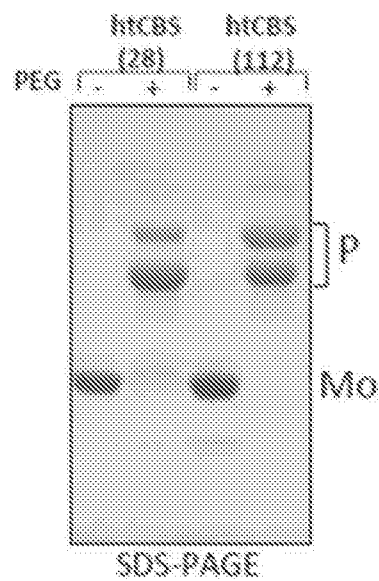


FIG. 5D

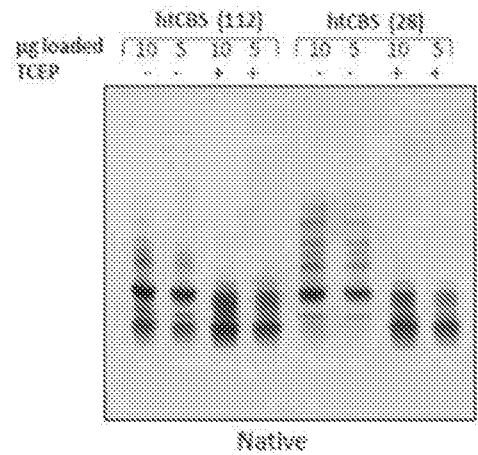


FIG. 5E

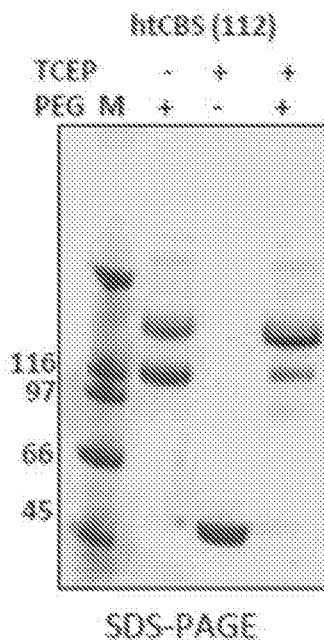


FIG. 5F

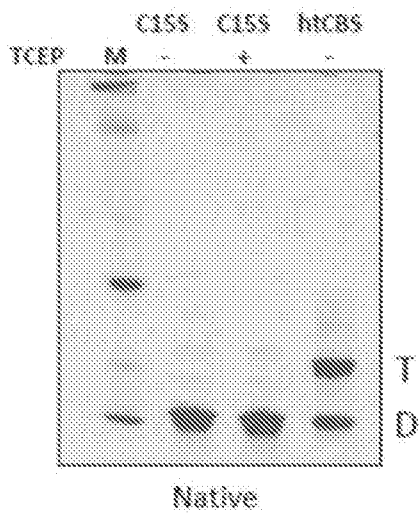


FIG. 5G

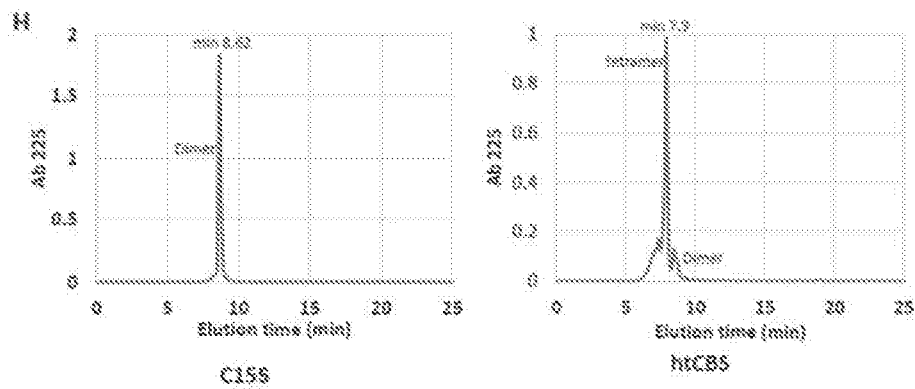


FIG. 5H

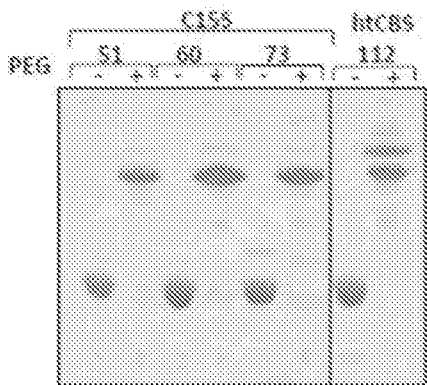


FIG. 6A

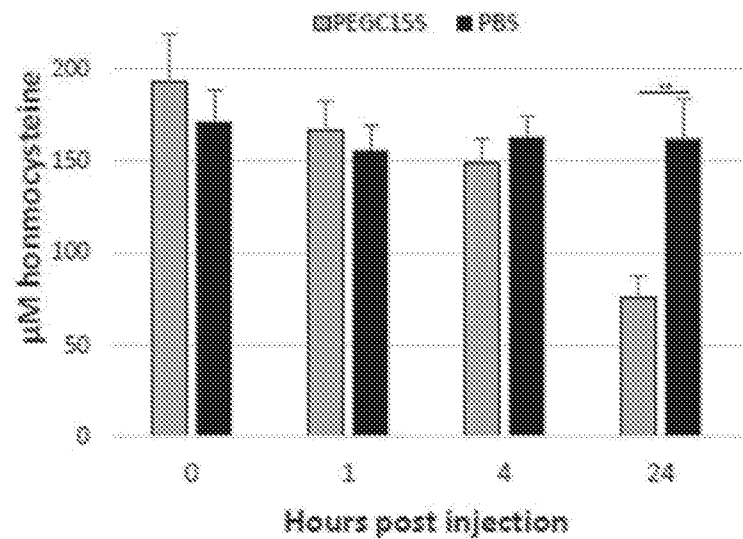


FIG. 6B

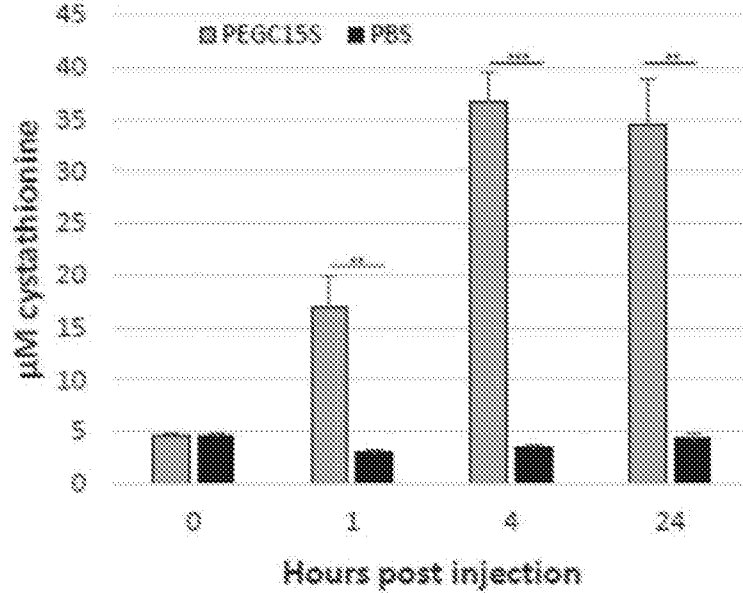


FIG. 6C

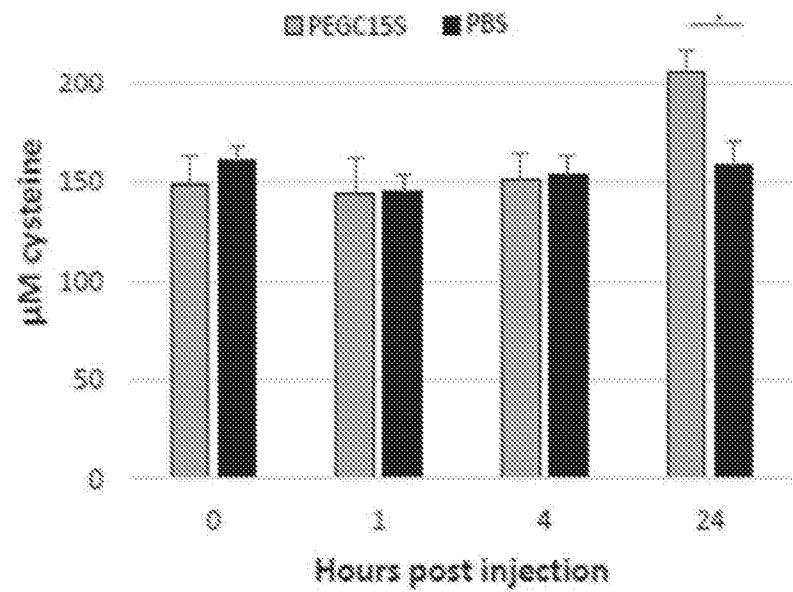


FIG. 7A

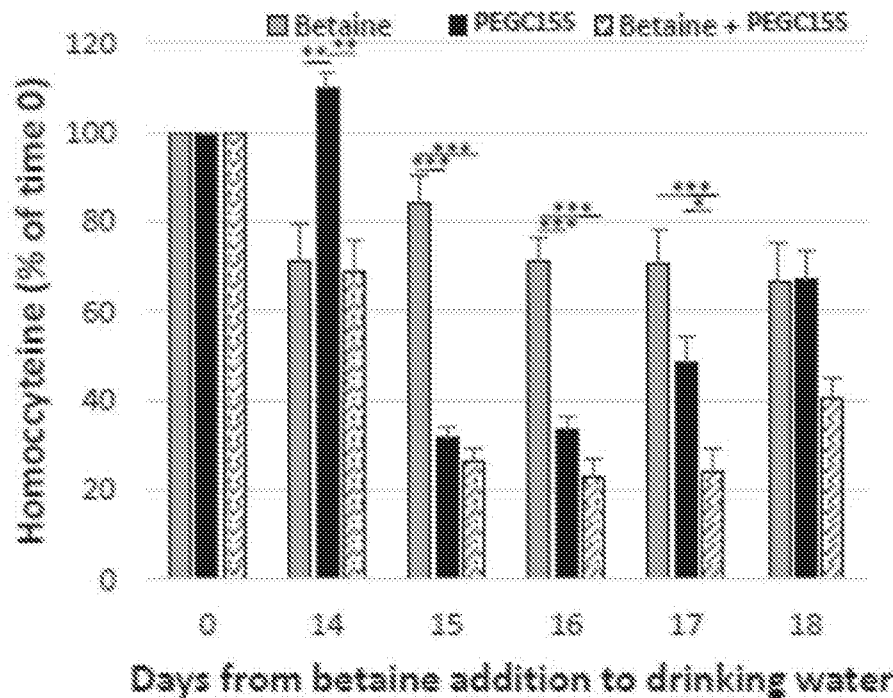


FIG. 7B

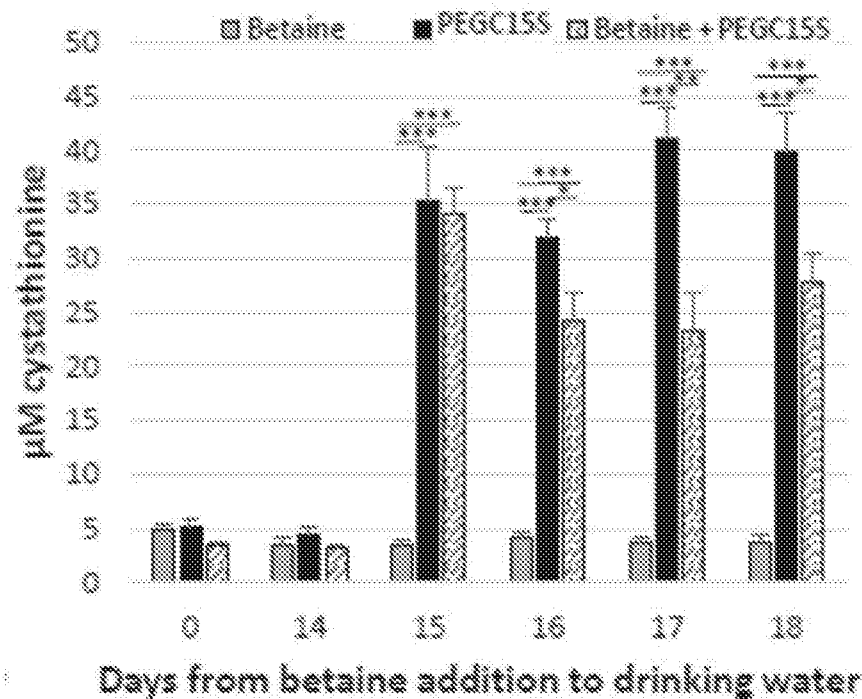


FIG. 8A

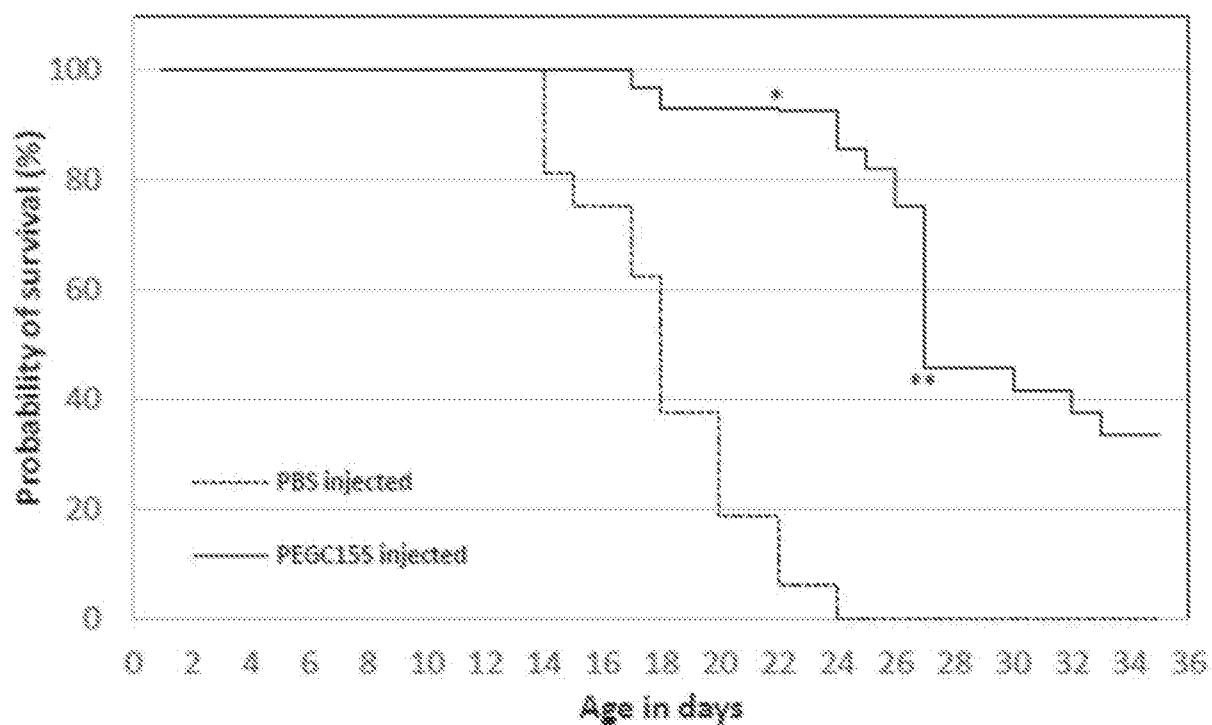


FIG. 8B

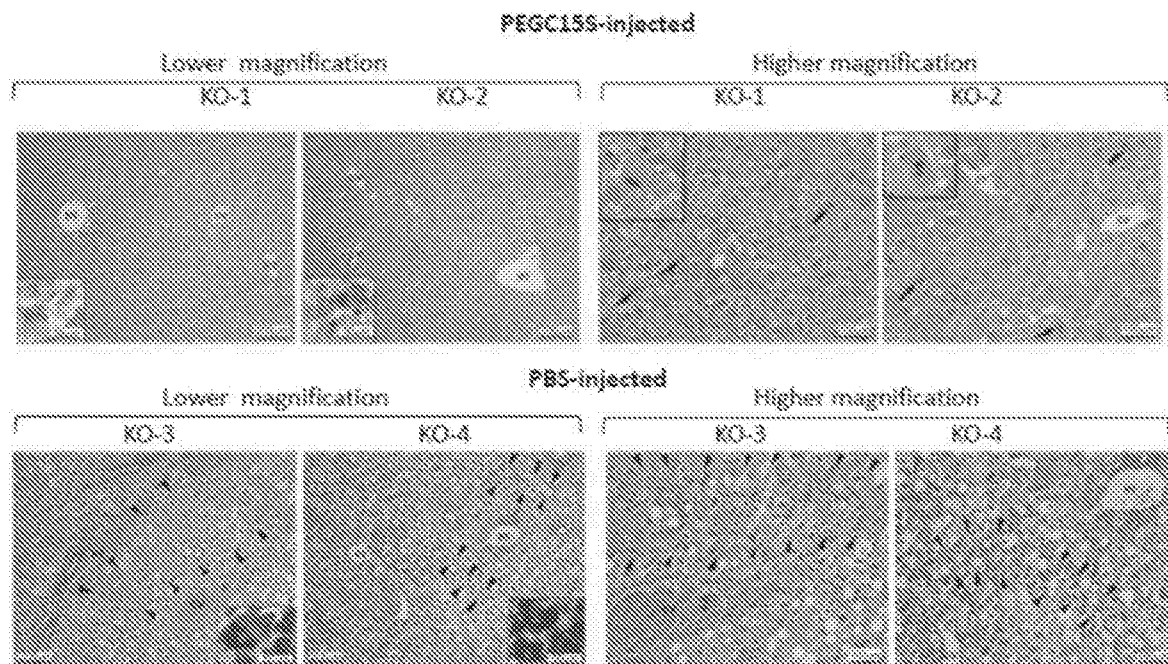


FIG. 9A



FIG. 9B

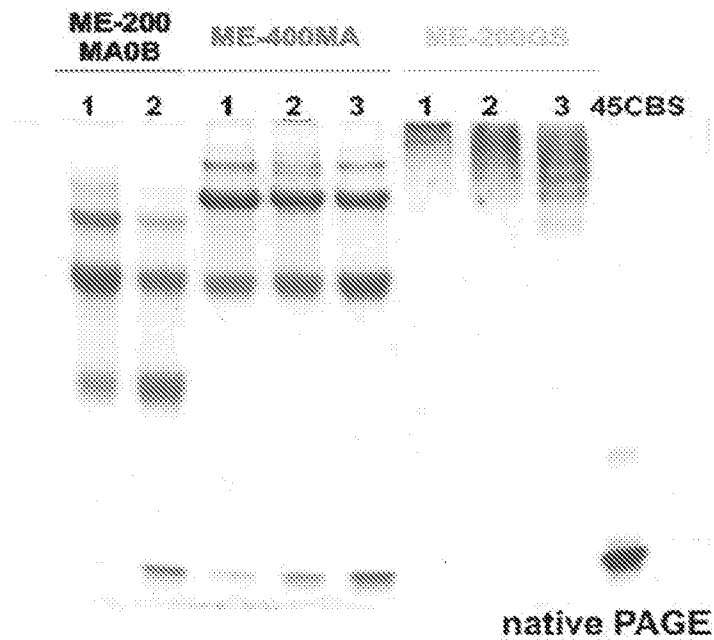


FIG. 10A

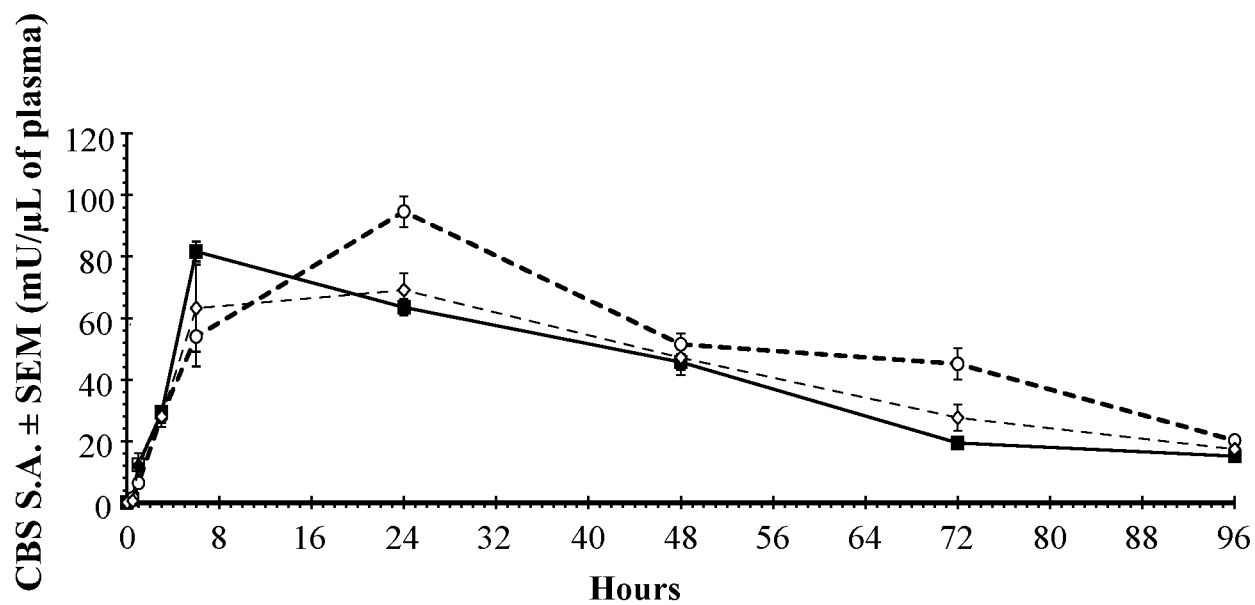


FIG. 10B

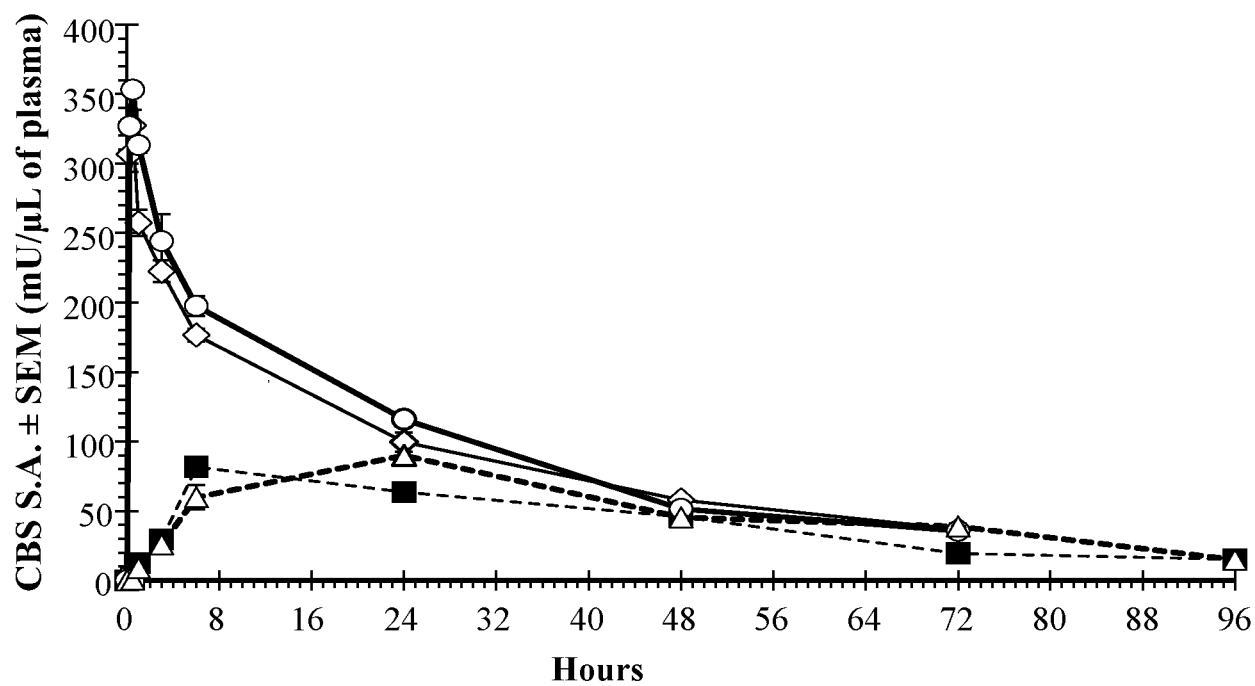


FIG. 10C

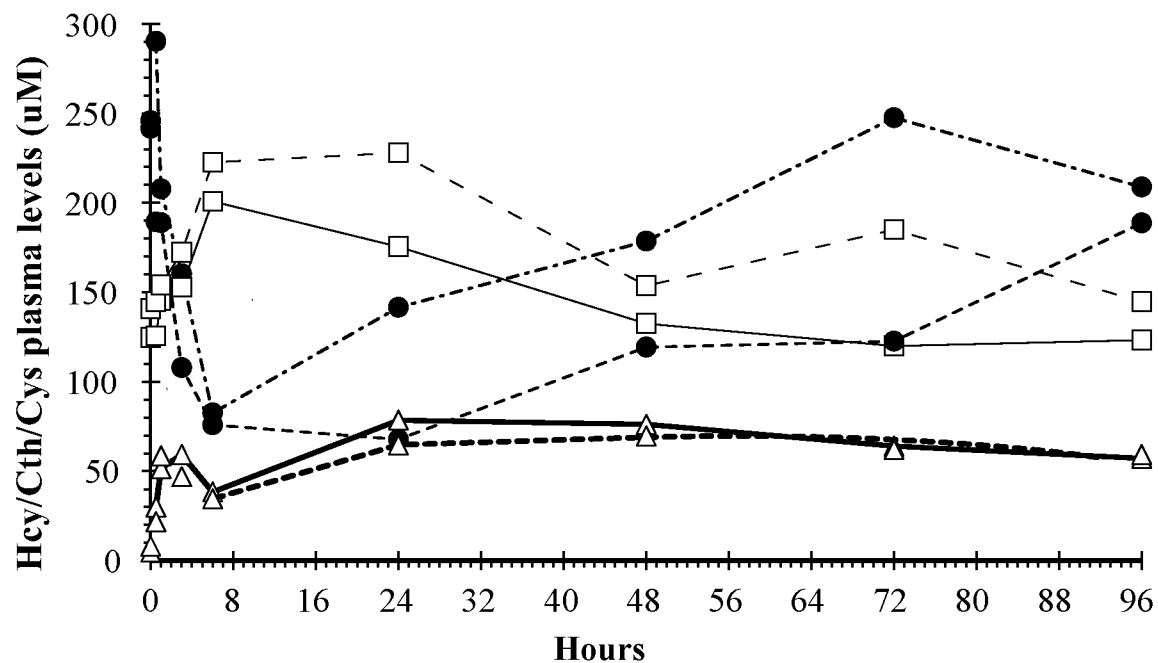


FIG. 10D

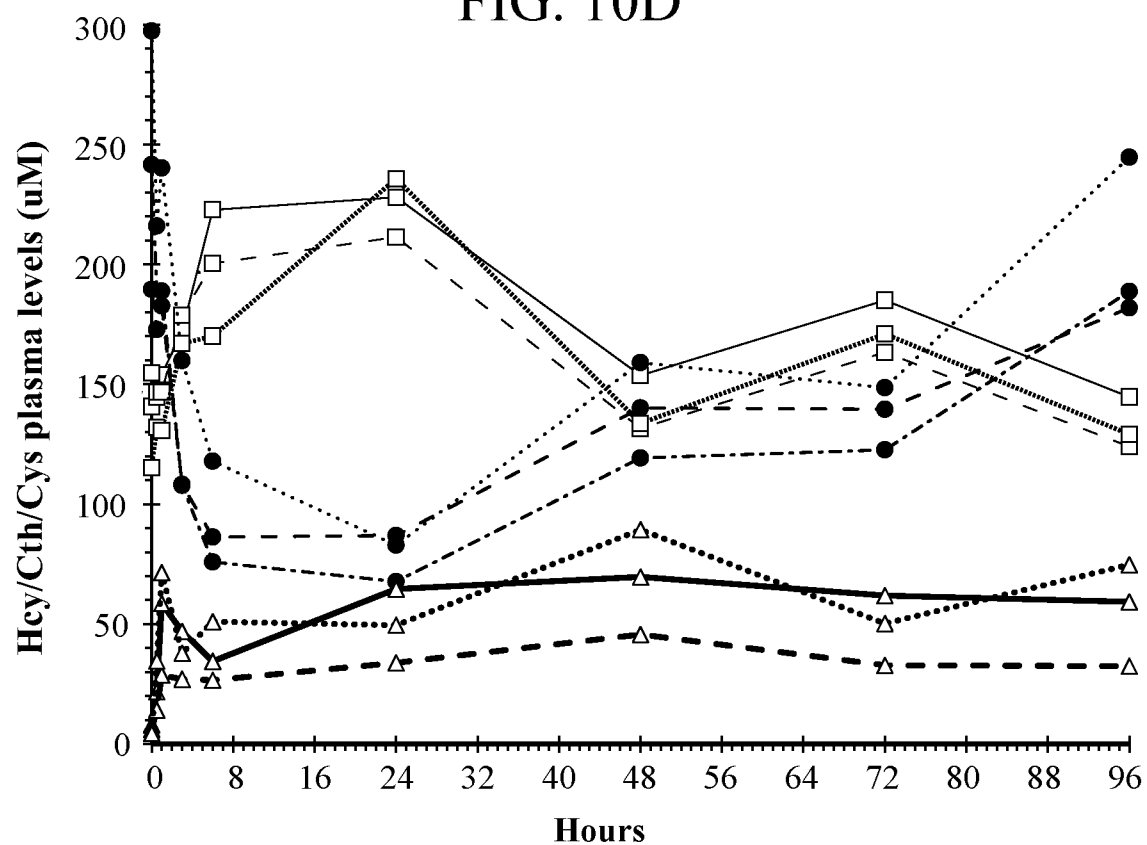
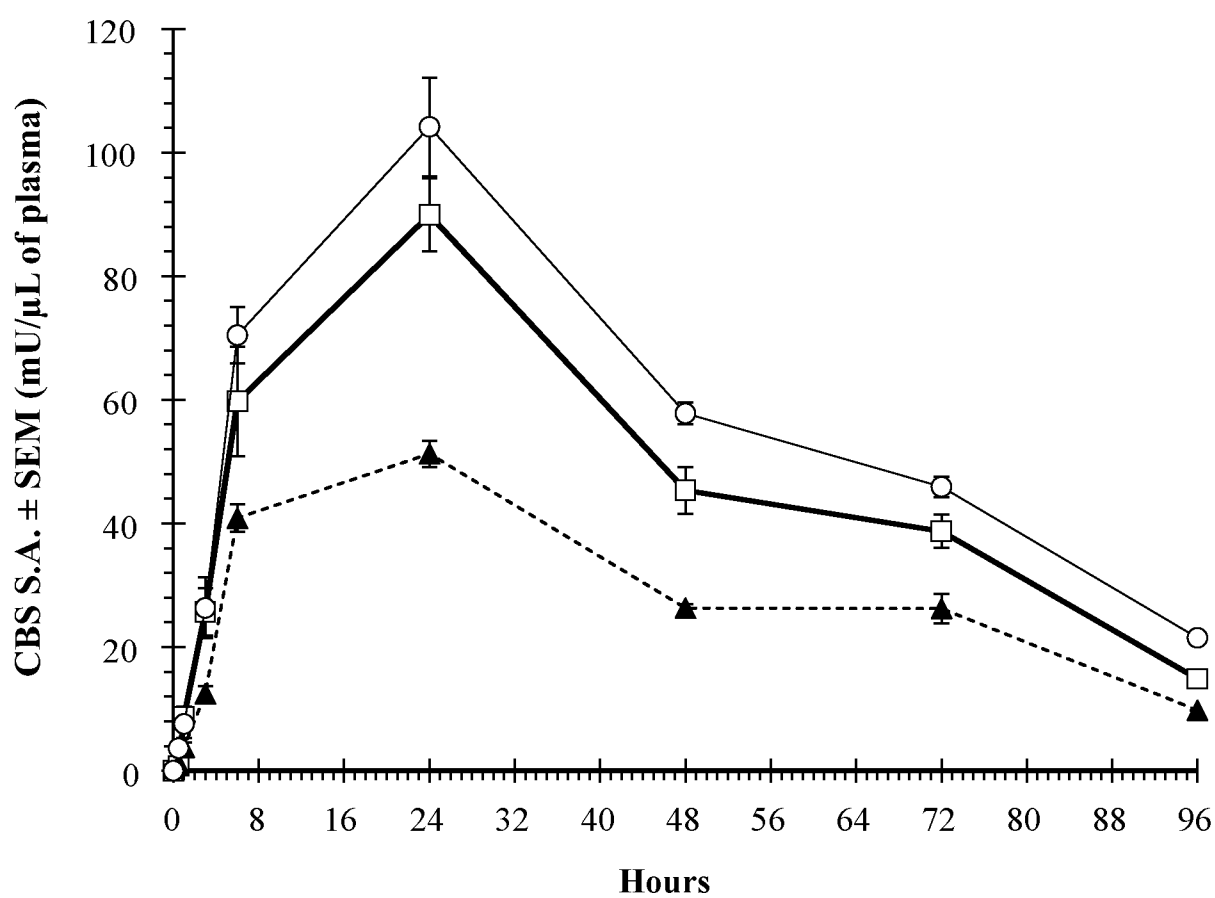
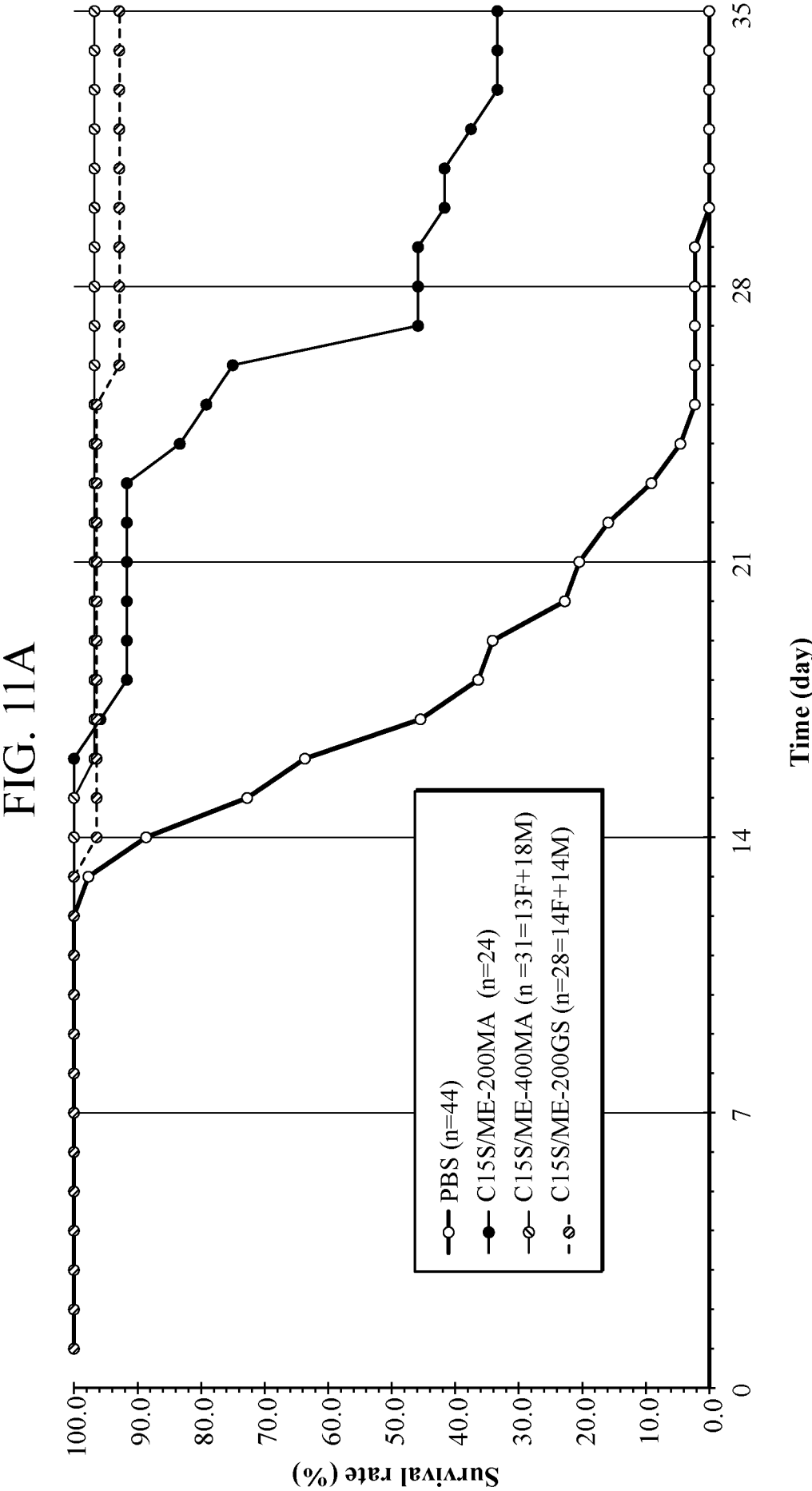
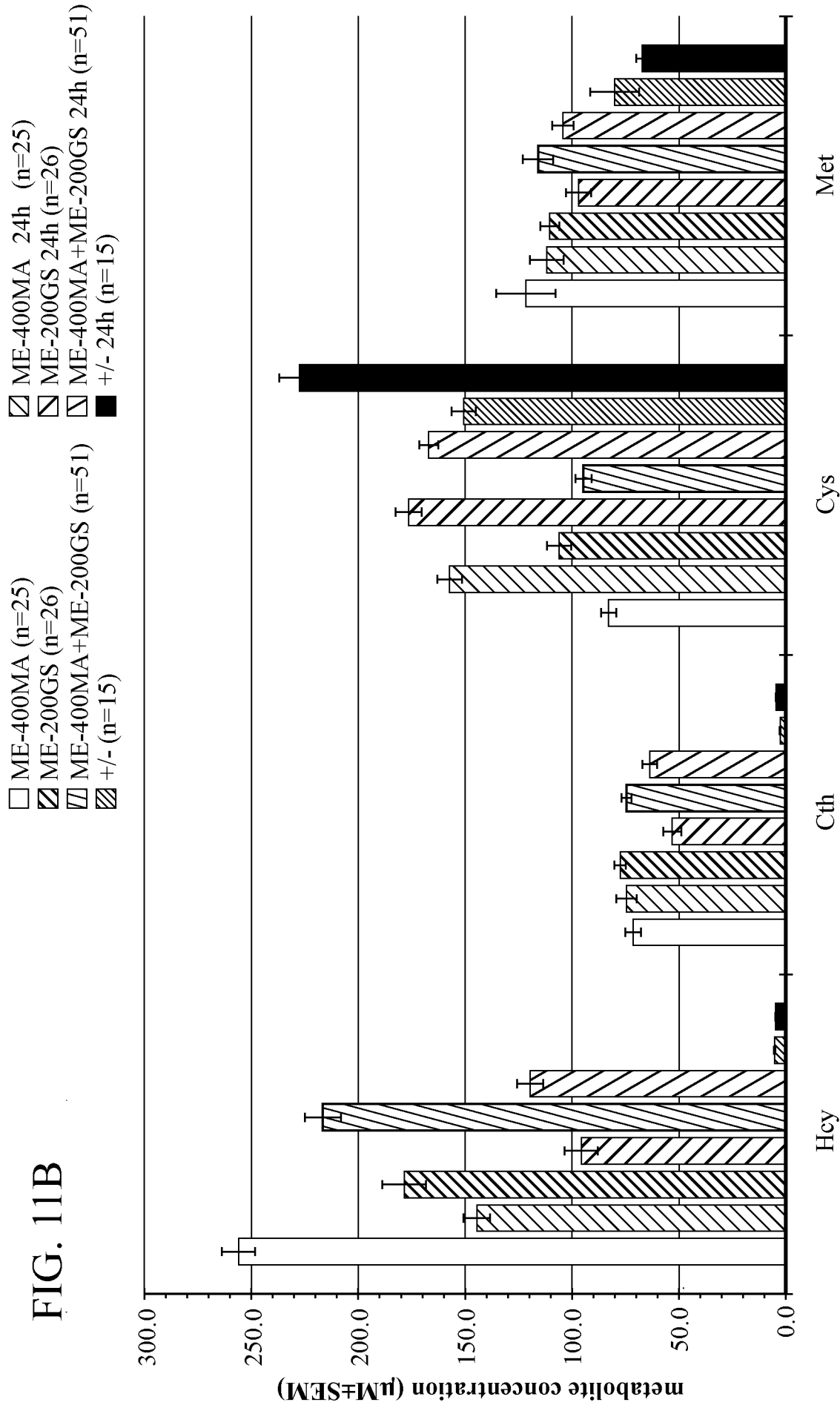


FIG. 10E







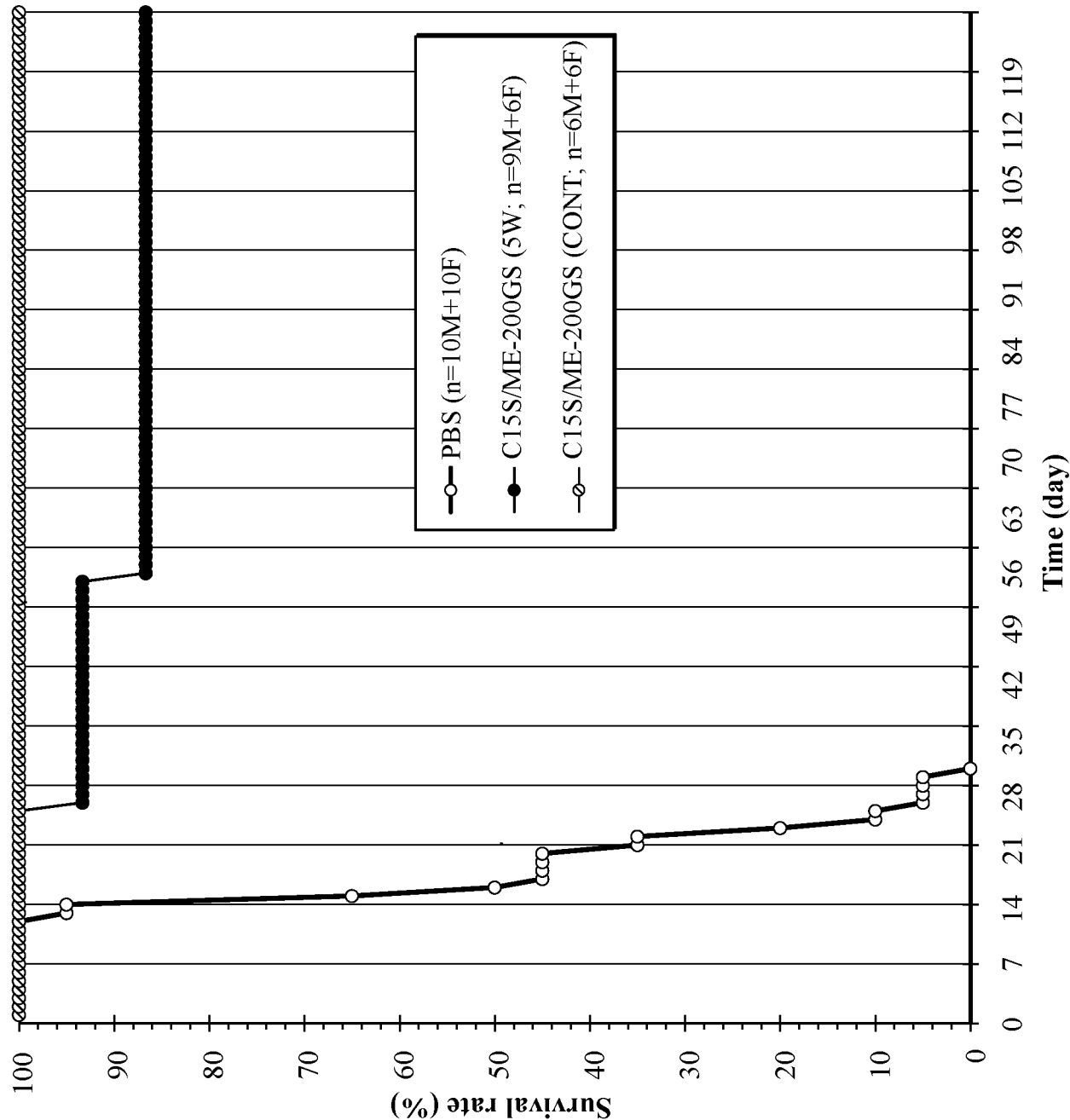
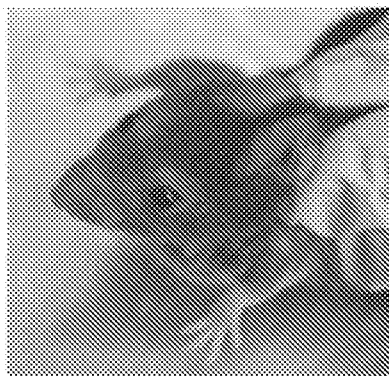
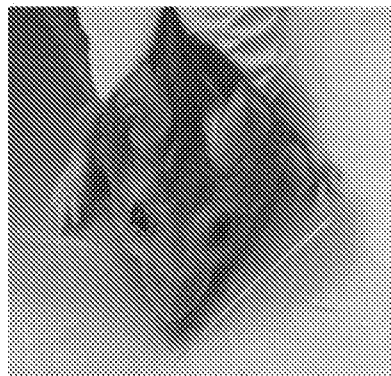


FIG. 11C

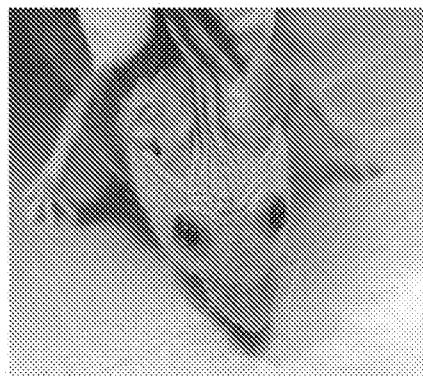
FIG. 12A



1739M (-/-)
6W treated
(4M)



1739M (-/-)
6W treated
(7M)



1739M (-/-)
6W treated
(9.5M)



1848M (-/-)
affected control
(7M)



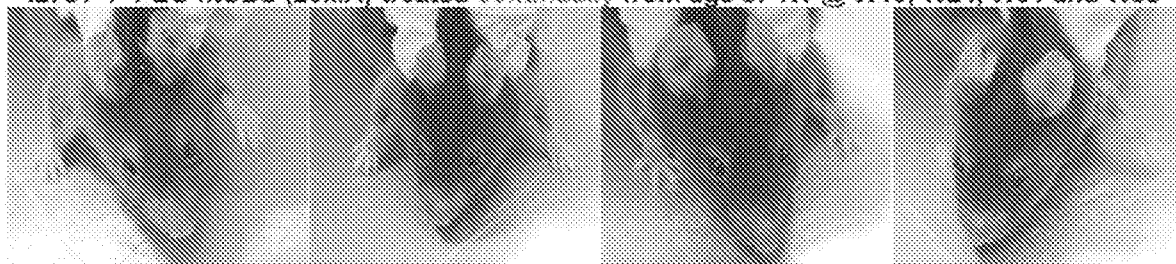
1737M (+/-)
healthy control
(9.5M)

FIG. 12B

I278T +/- untreated @ W19 and W34



I278T -/- PEG htCBS (20MA)-treated continually from age of 4W @ W19, W24, W31 and W35



I278T -/- untreated @ W38



FIG.12C

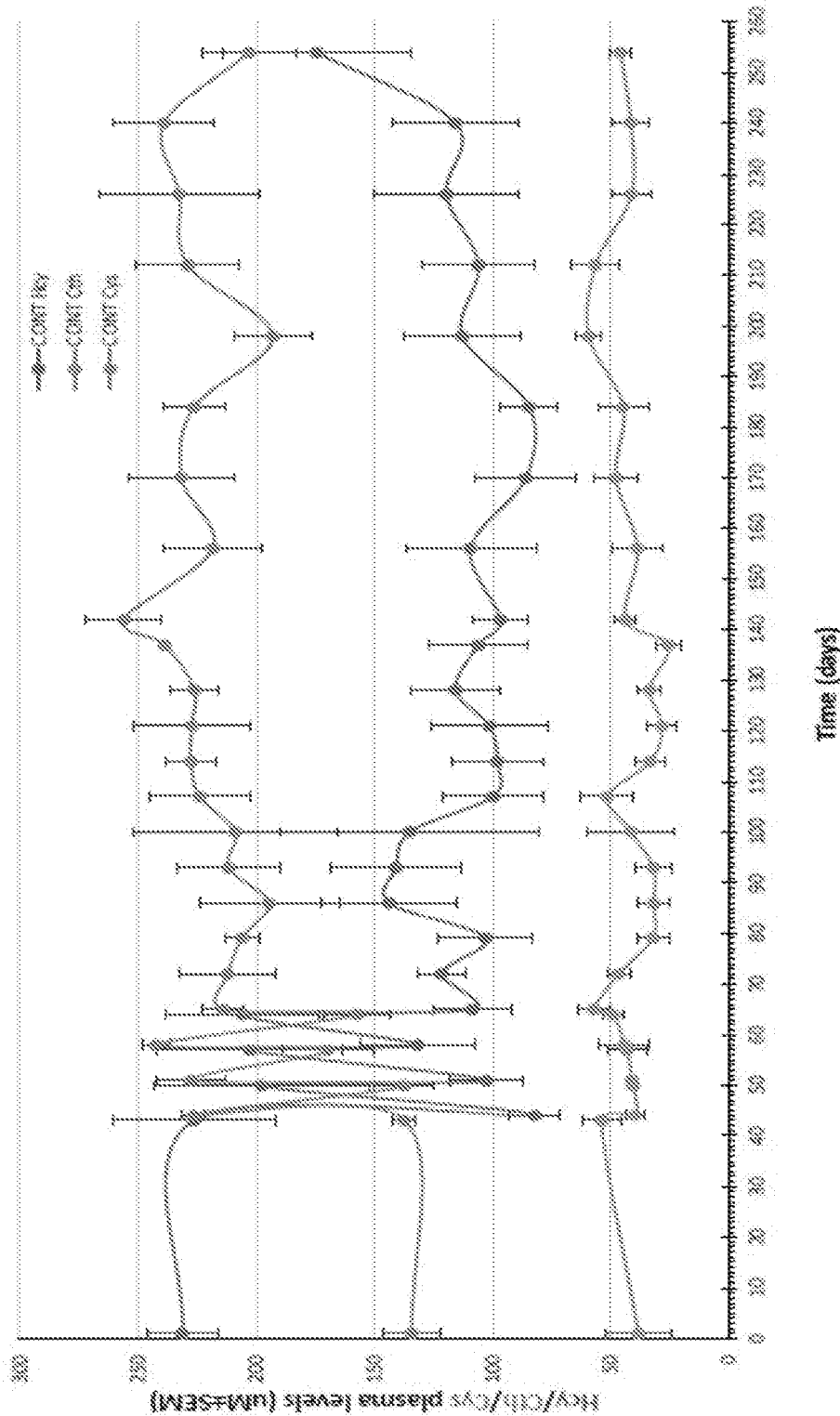
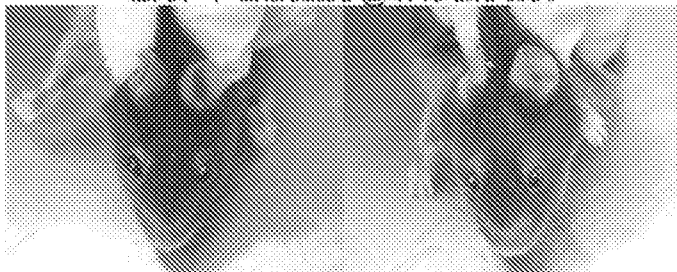
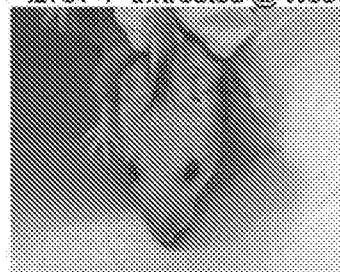


FIG. 12D

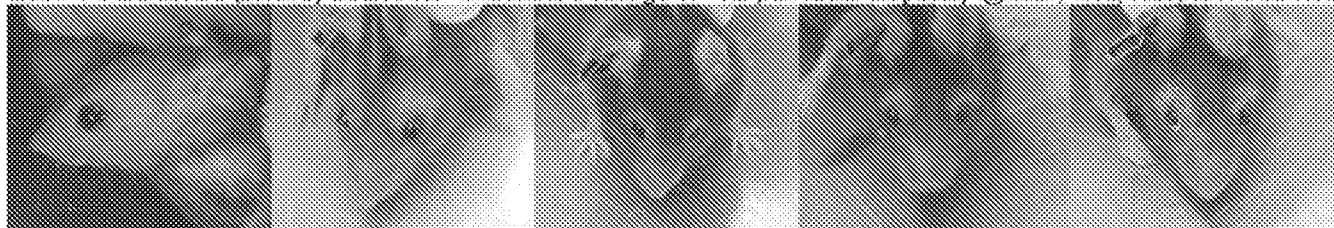
I278T +/- untreated @ W19 and W34



I278T +/- untreated @ W38



I278T +/- PEG hiCBS (20NHS)-treated for ~6 months from age of ~6M (full facial alopecia) @ W27, W32, W37, W44 and W50



I278T +/- PEG hiCBS (40NHS)-treated for ~6 months from age of ~6M (full facial alopecia) @ W27, W32, W37, W44 and W50

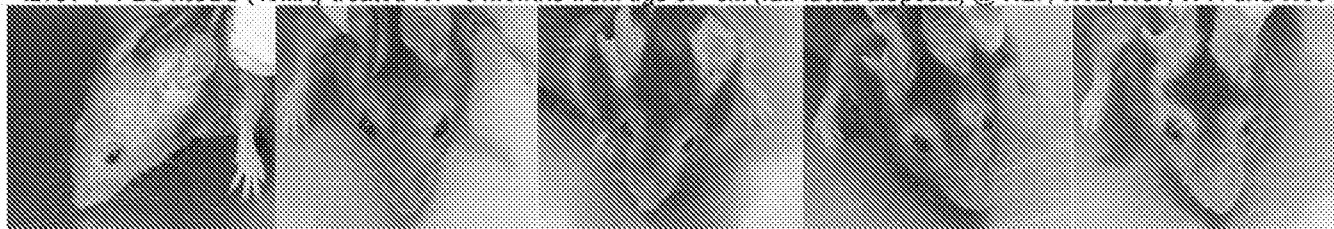


FIG. 12E

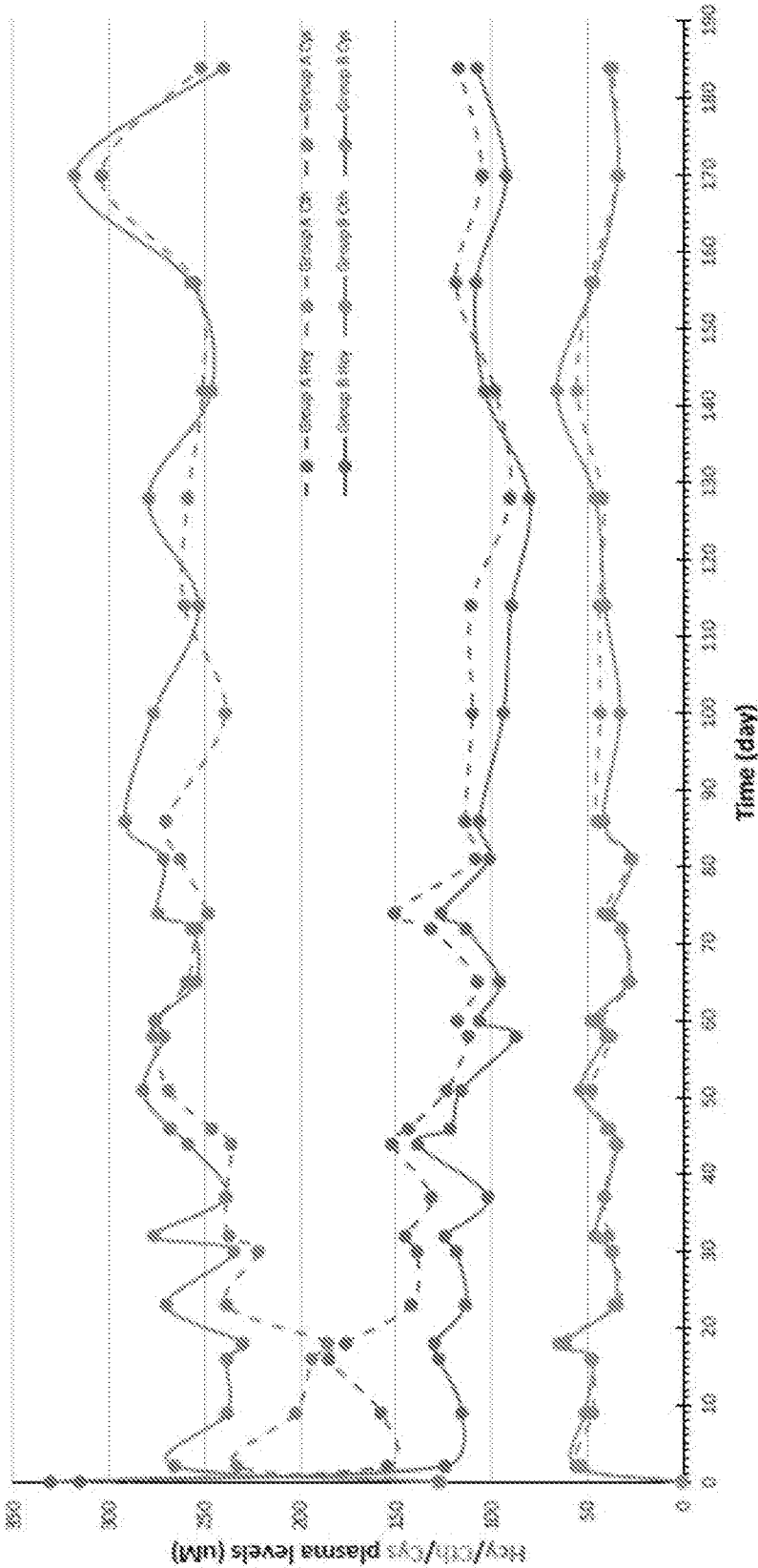
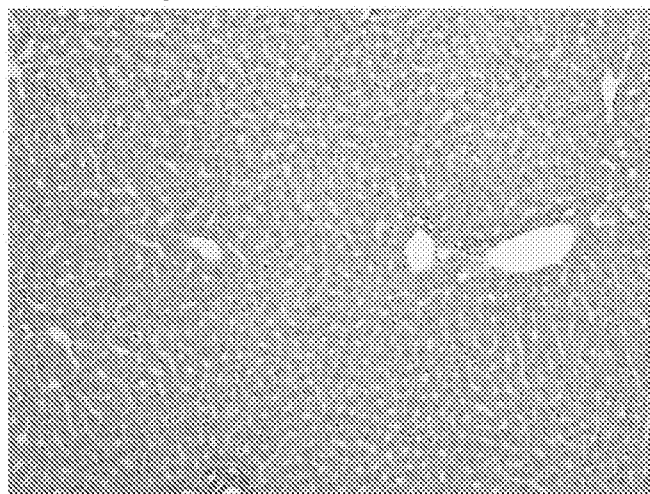


FIG. 13A

**KO1982 (-/-) PBS injected MODERATE
TO SEVERE HEPATOPATHY**



**KO2276 (-/-) 20NHS C15S/ME-200GS
injected MILD CHANGES**



**KO1985 (+/-) healthy control
MINIMAL CHANGES**

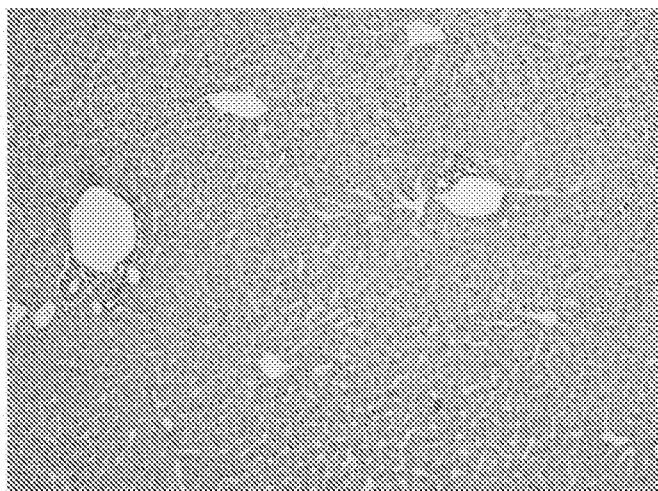
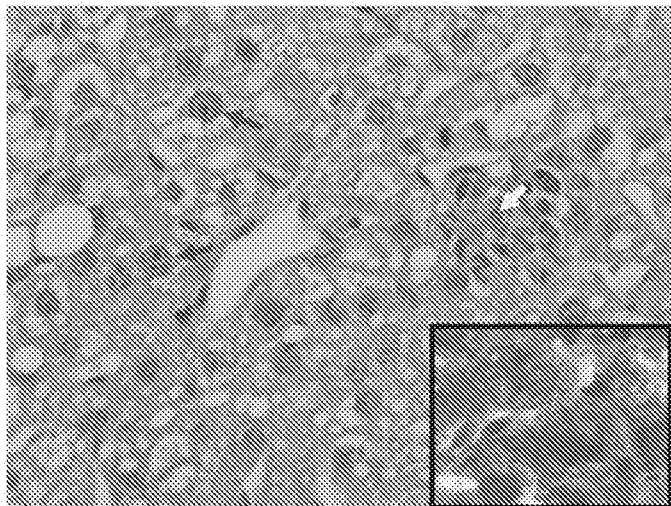
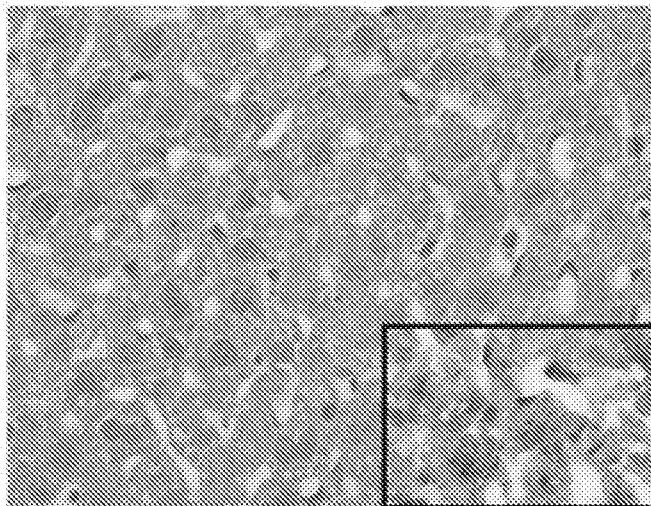


FIG. 13B

KO1982 (-/-) PBS injected MODERATE
TO SEVERE HEPATOPATHY



KO2276 (-/-) 20NHS PEG-CBS injected MILD CHANGES



KO1985 (+/-) healthy control MINIMAL CHANGES

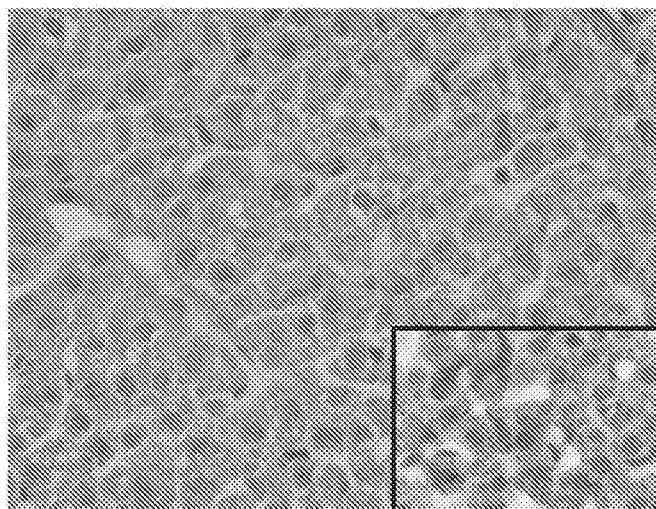


FIG. 14A

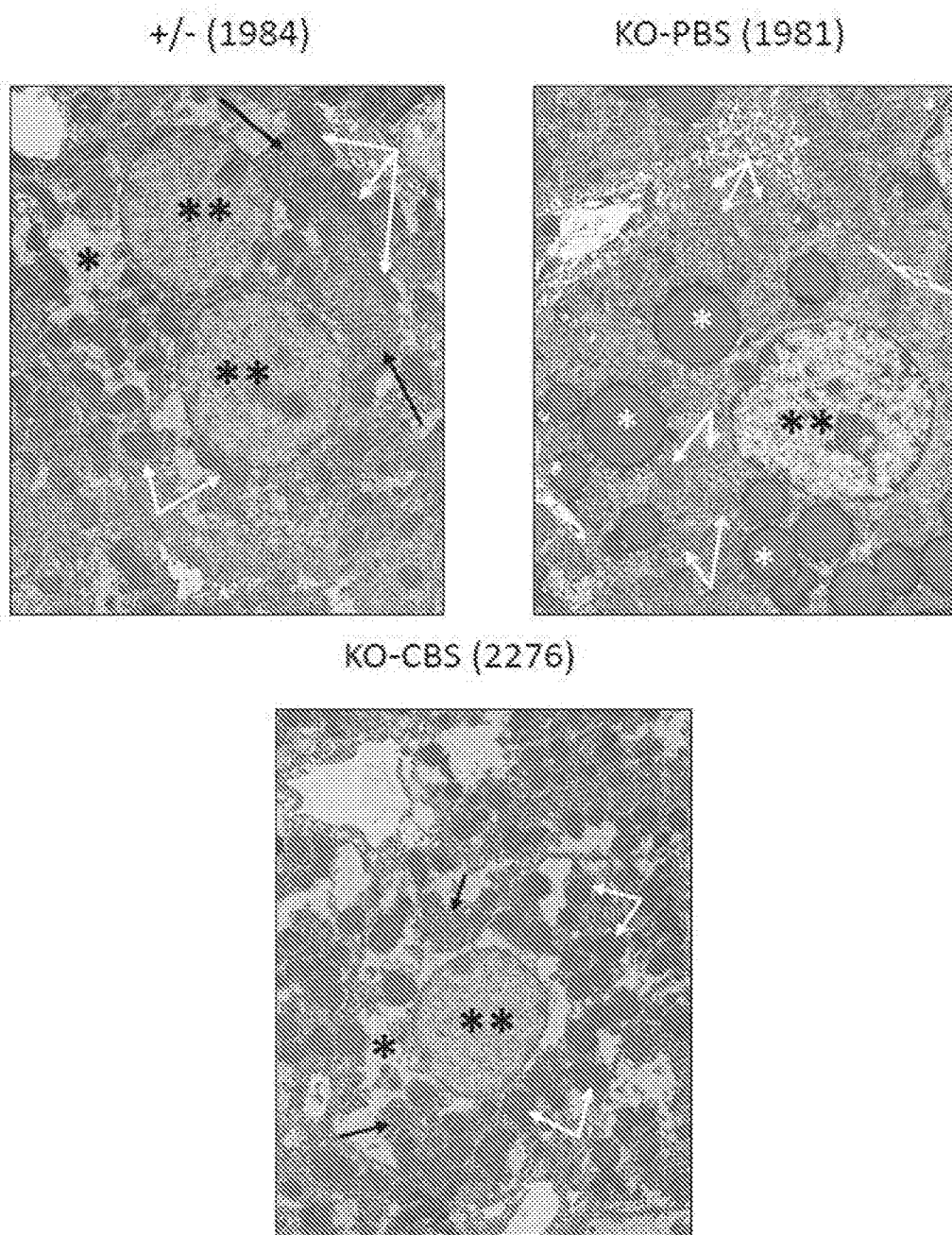


FIG. 14B

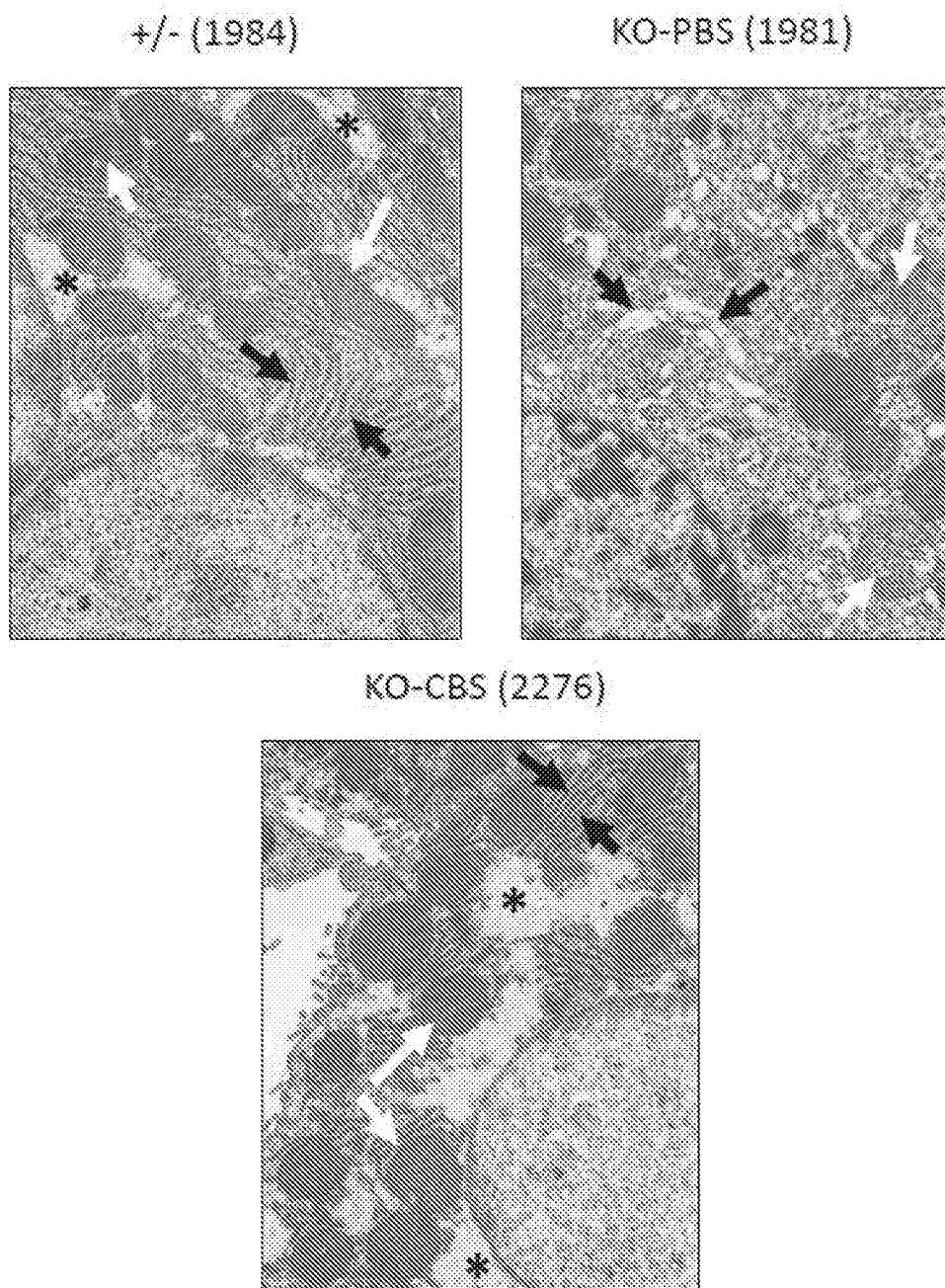


FIG. 15A

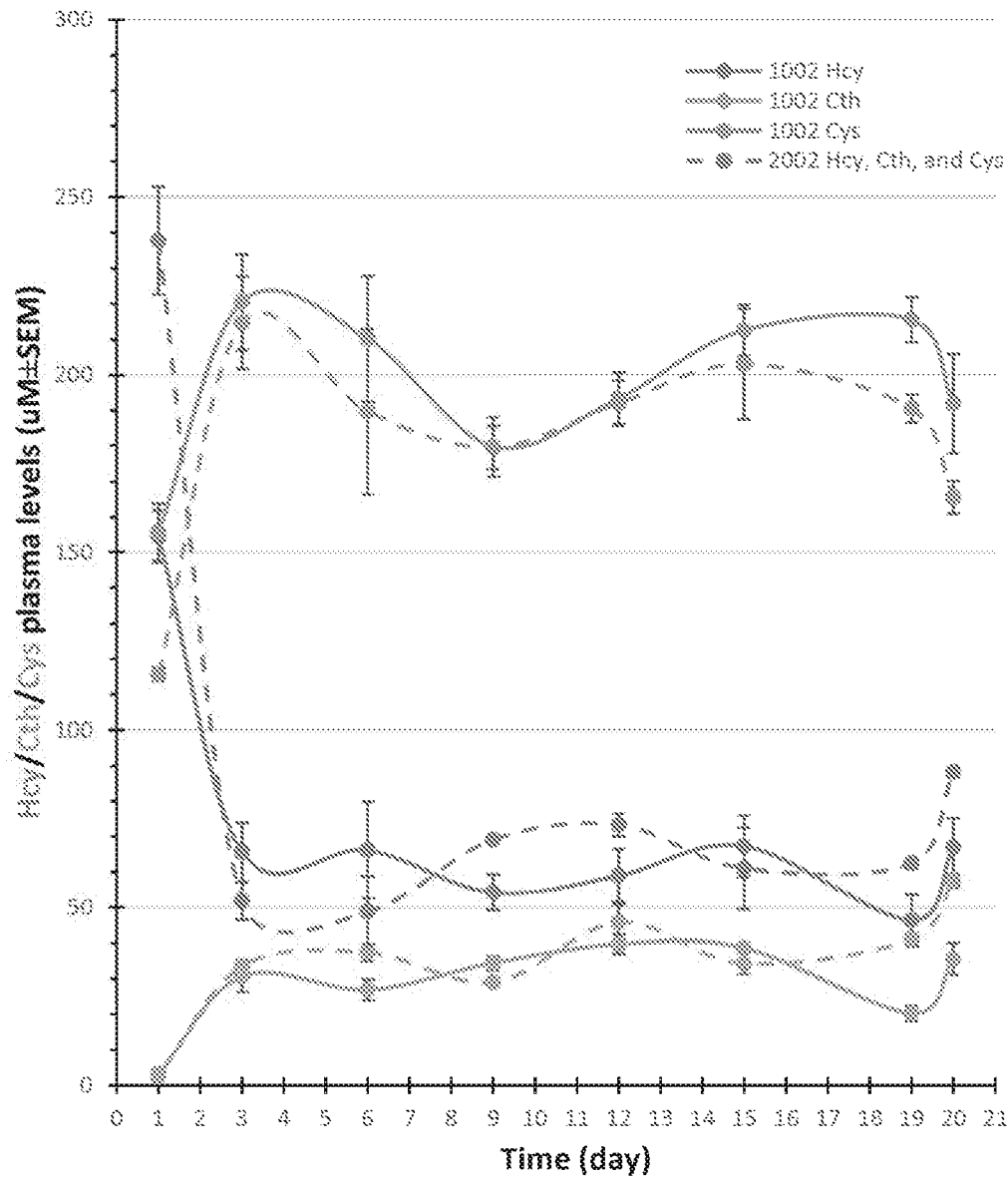


FIG. 15B

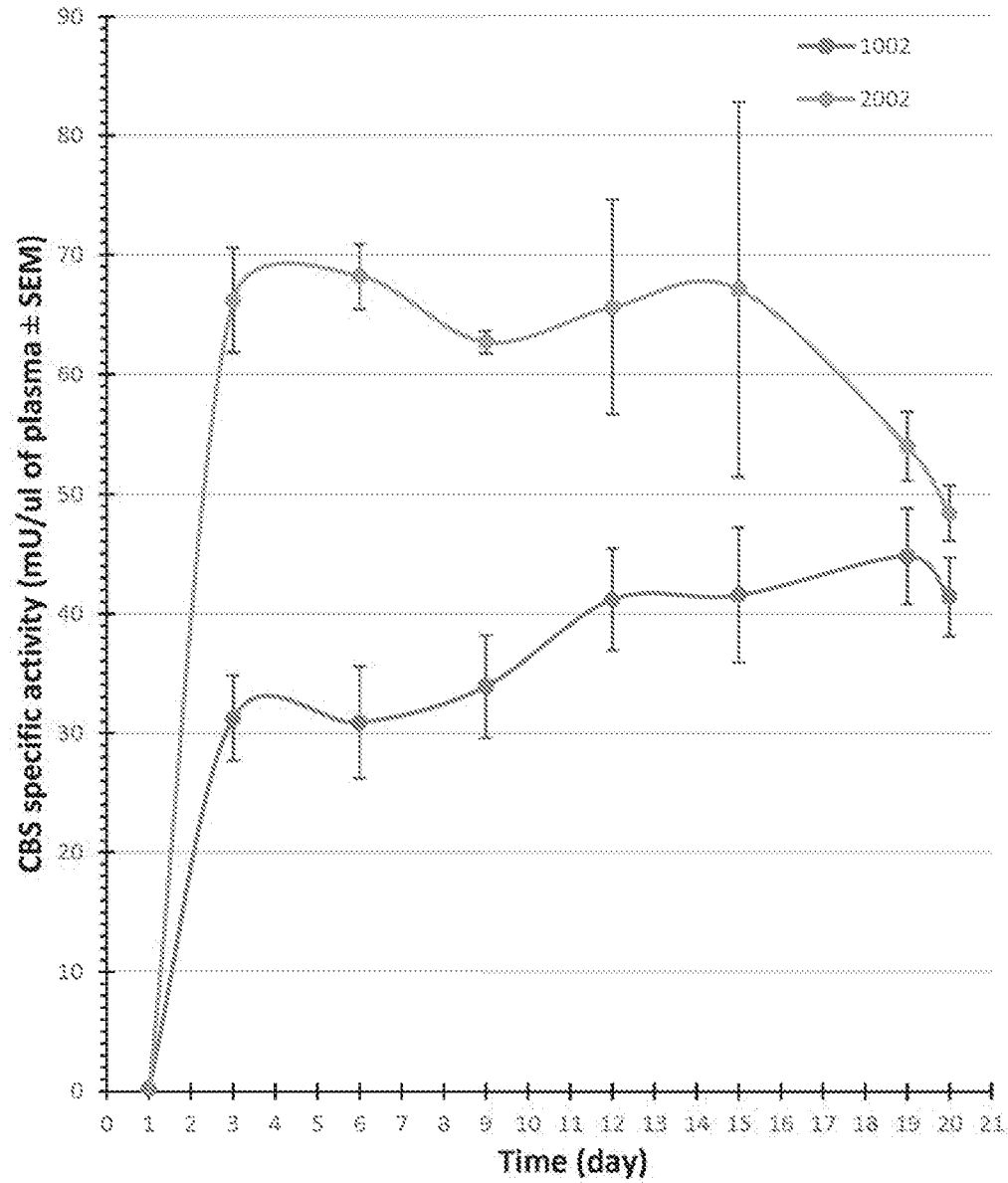


FIG. 15C

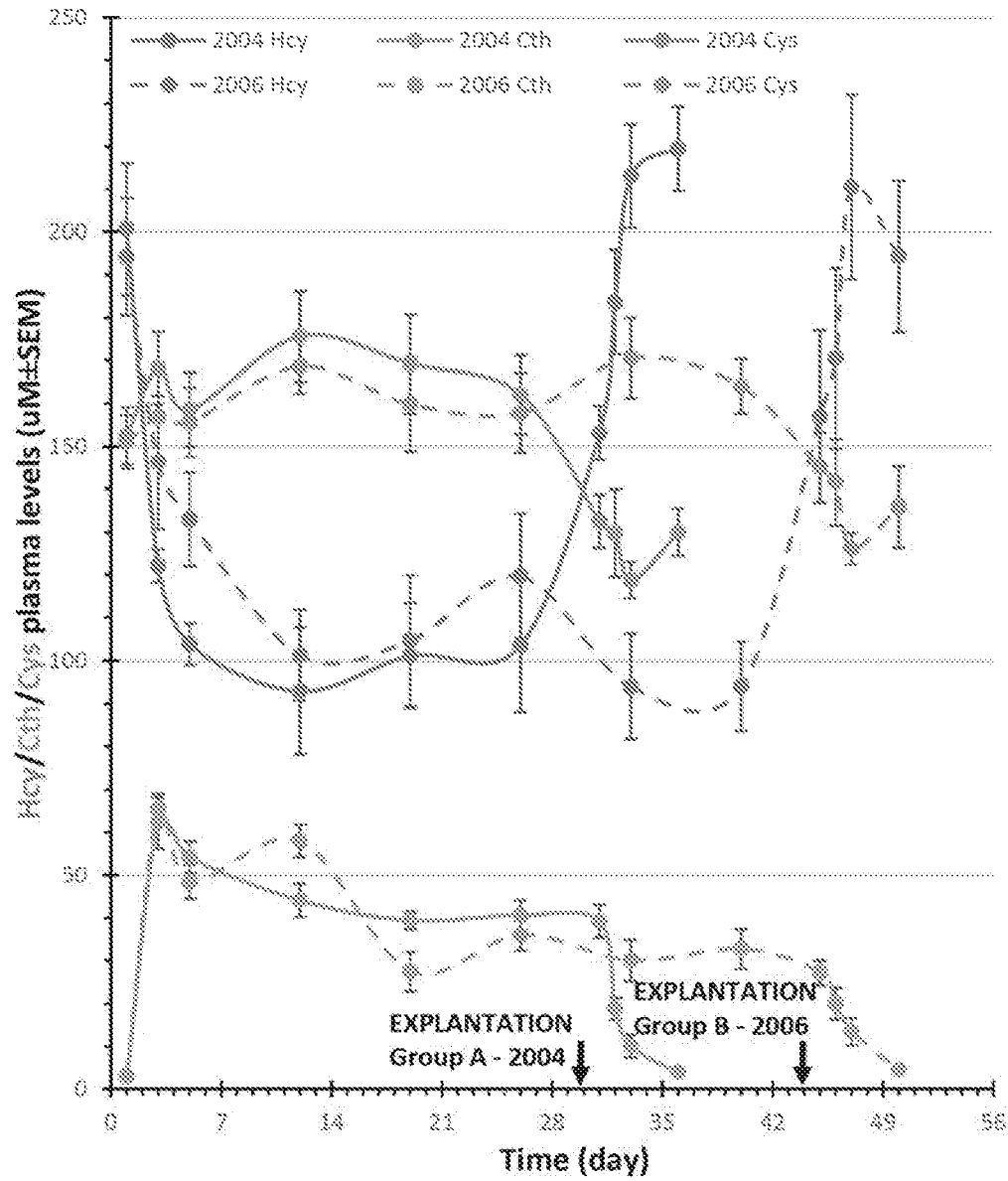
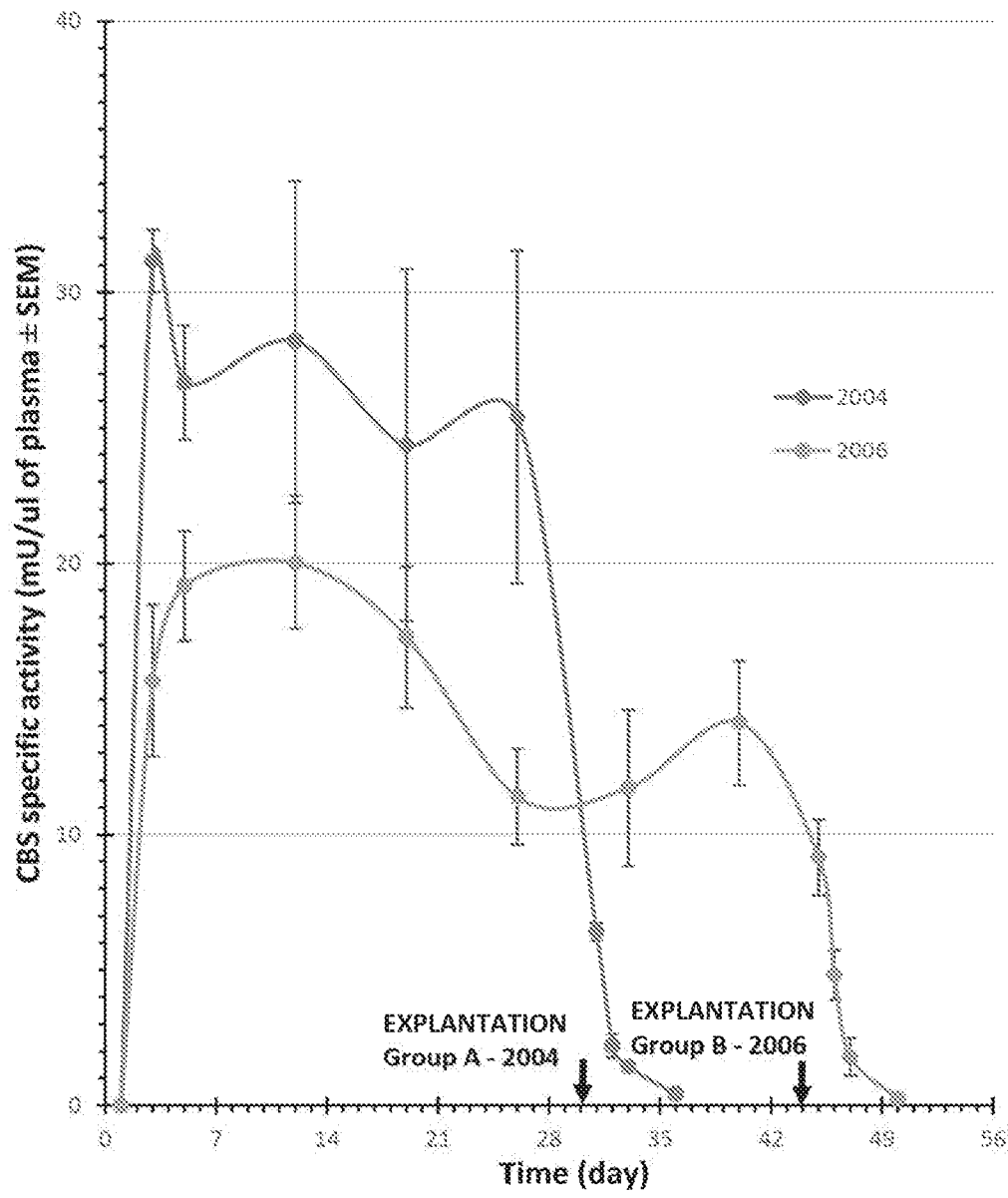


FIG. 15D



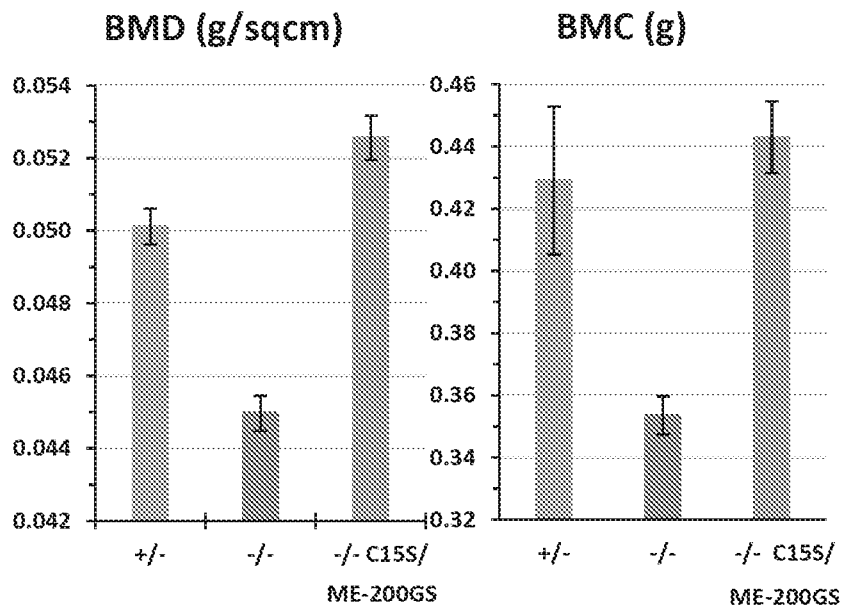


FIG. 16A

FIG. 16B

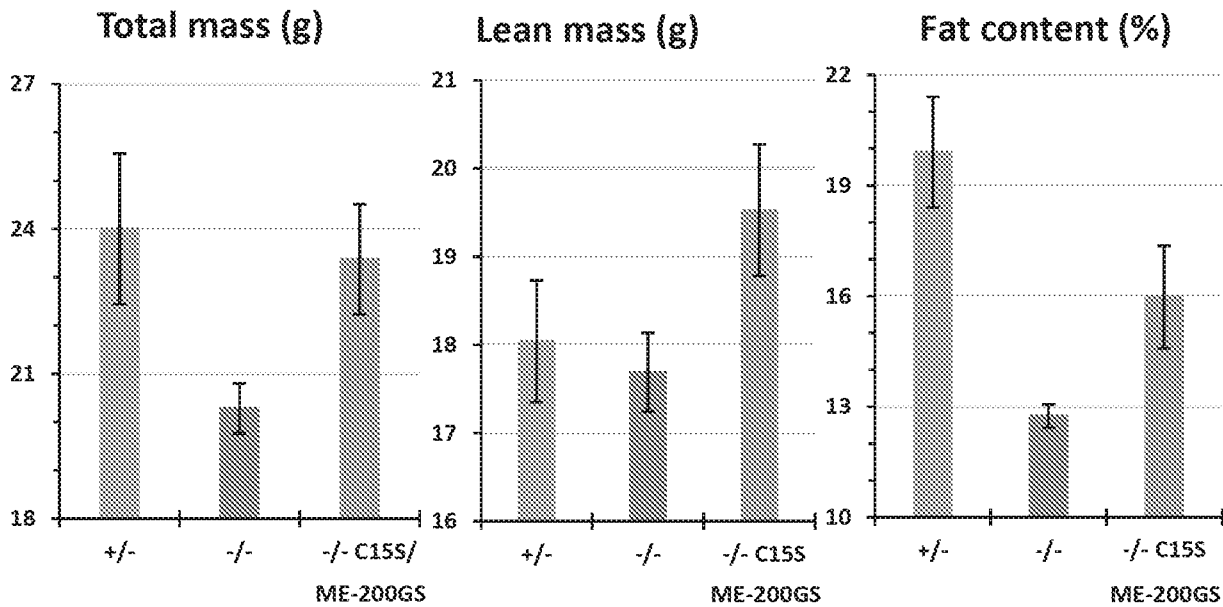


FIG. 16C

FIG. 16D

FIG. 16E

FIG. 16F

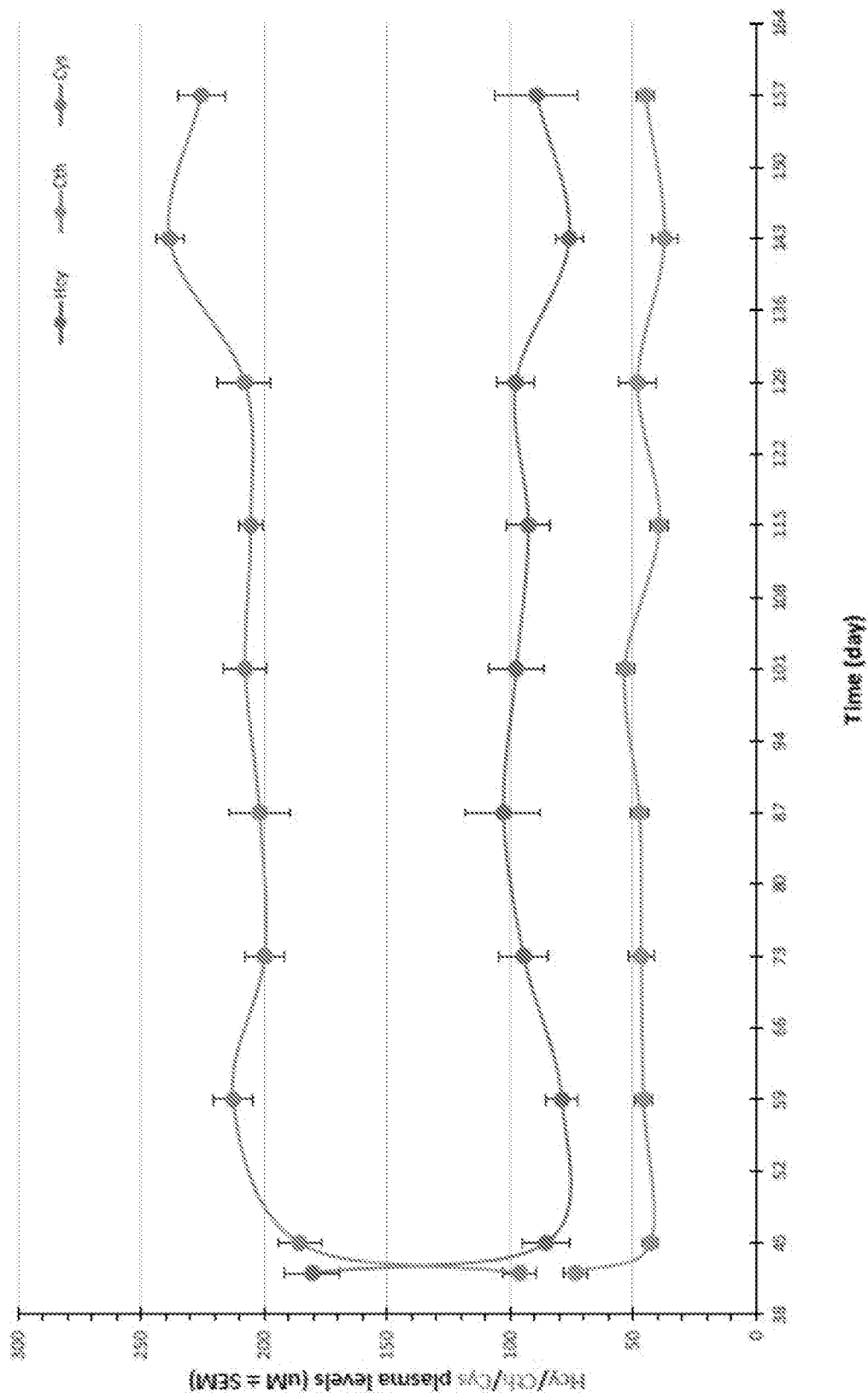
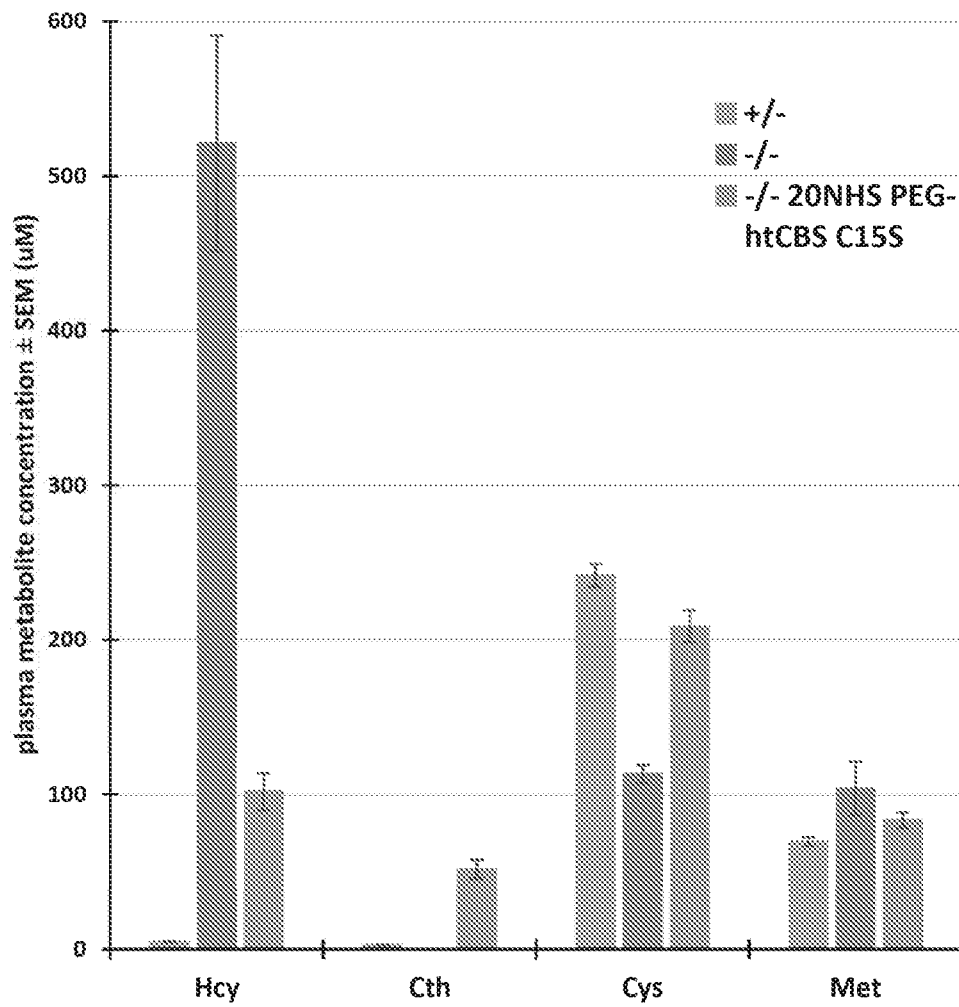


FIG. 16G



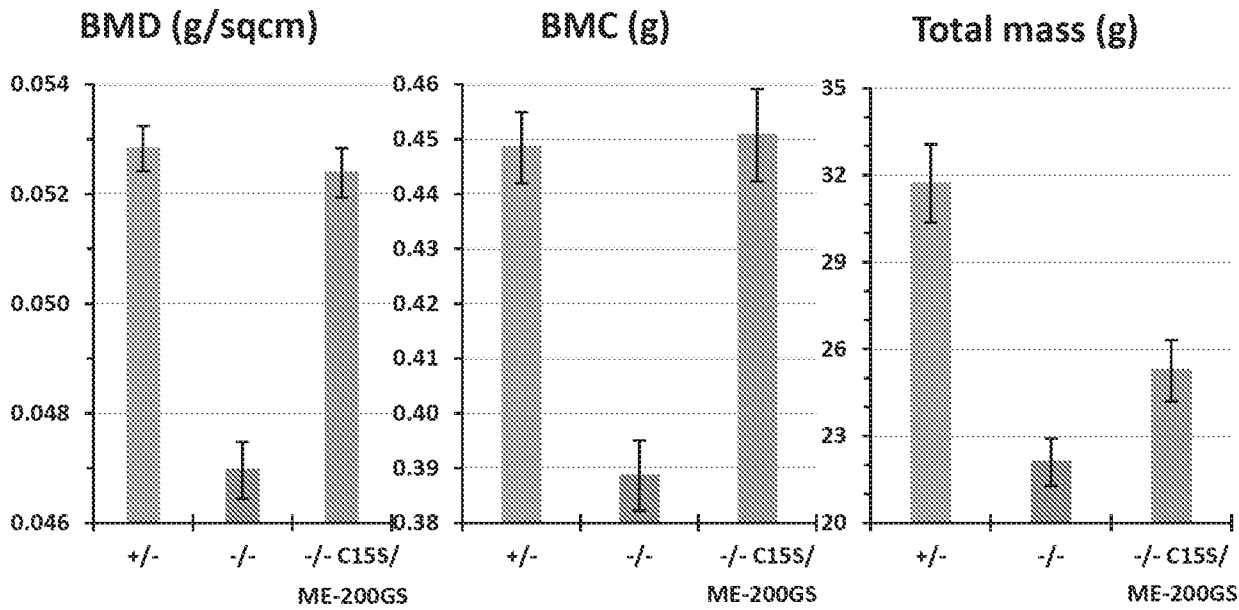


FIG. 17A

FIG. 17B

FIG. 17C

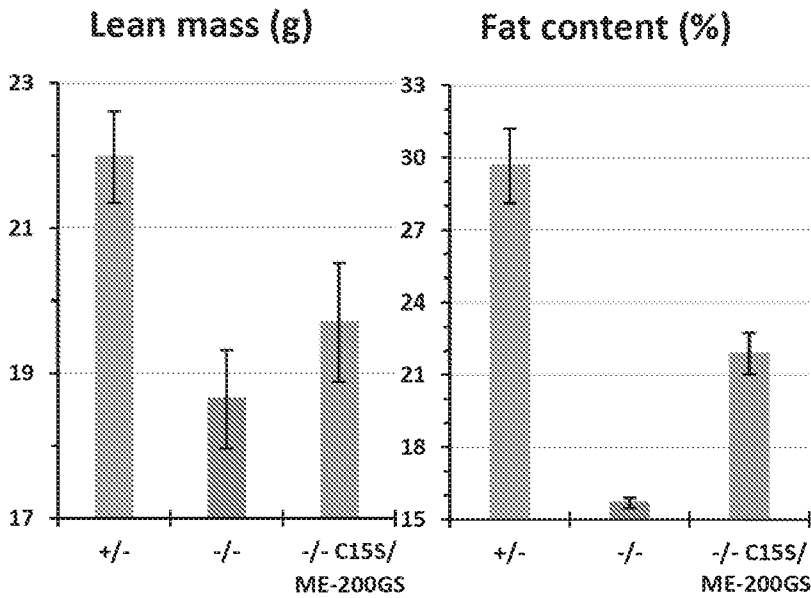


FIG. 17D

FIG. 17E

FIG. 17F

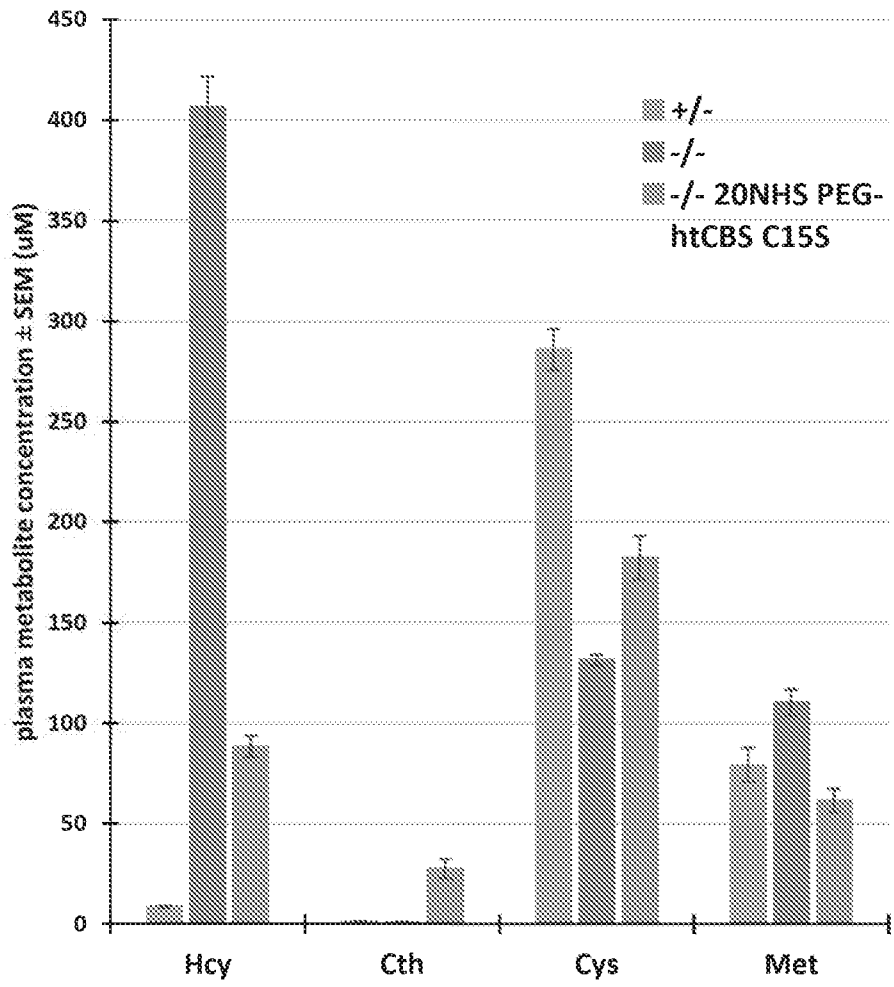


FIG. 18A

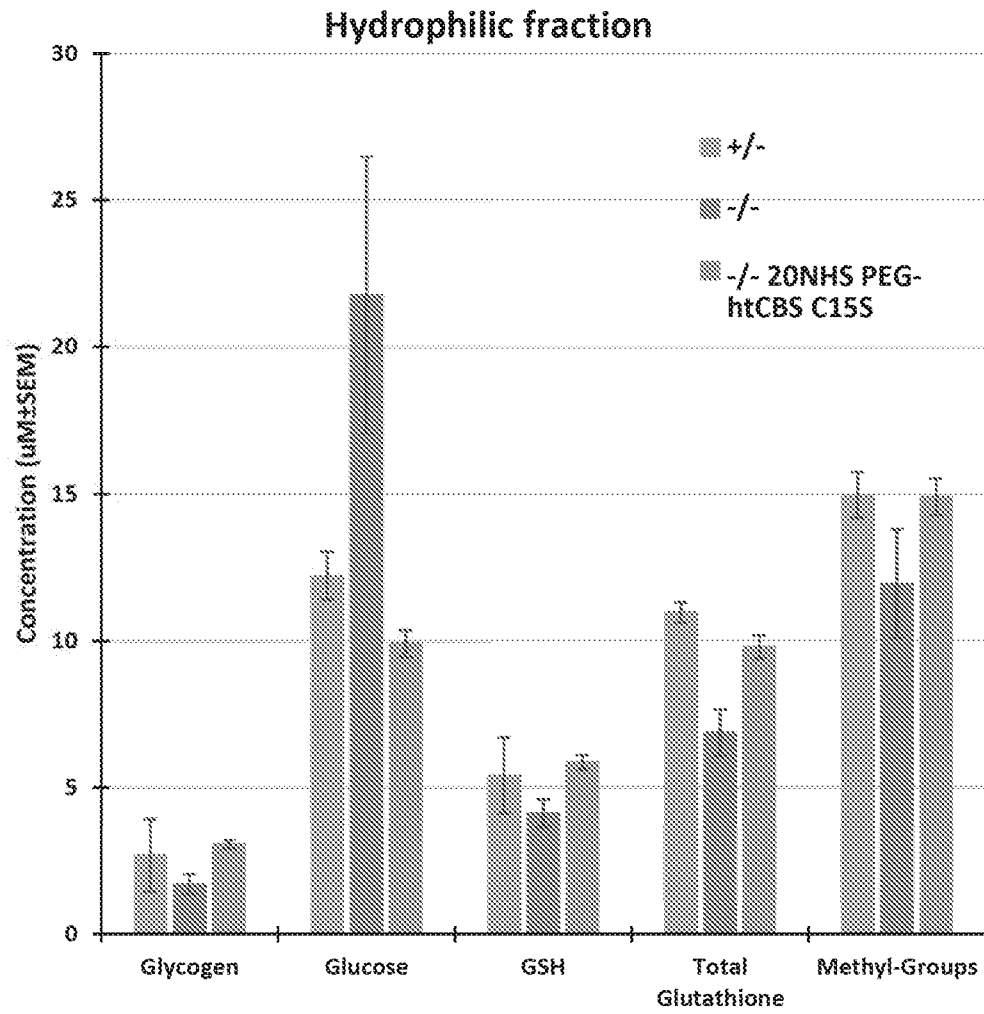


FIG. 18B

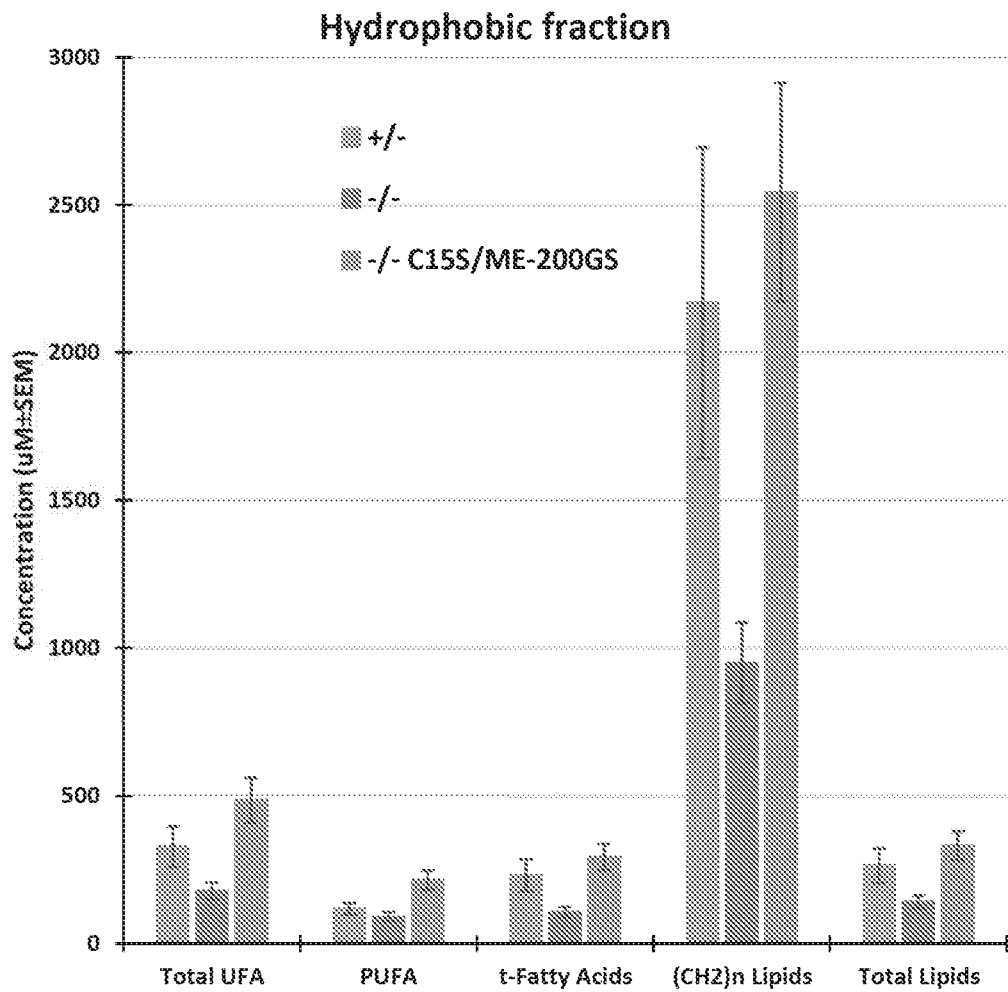
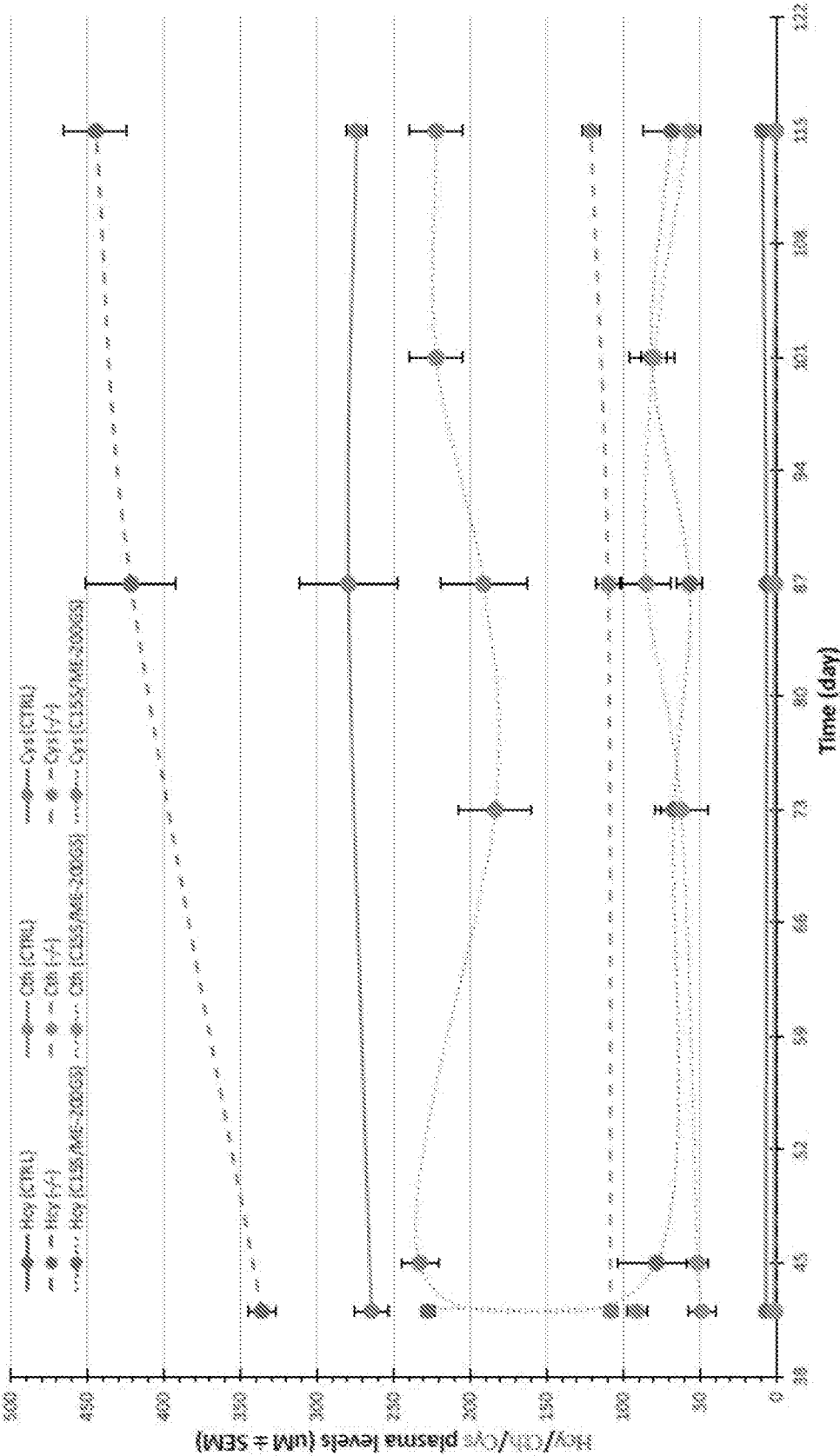


FIG. 19A



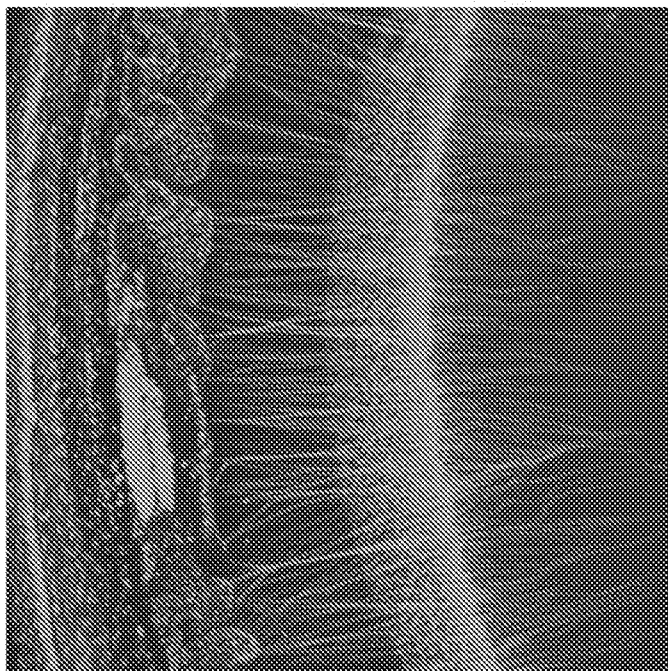


FIG. 19B

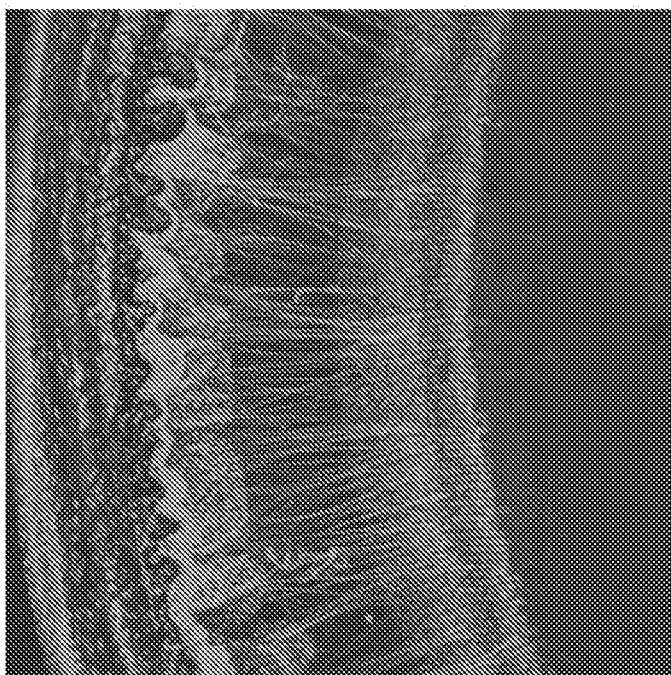


FIG. 19C



FIG. 19D



FIG. 19E

FIG. 20A

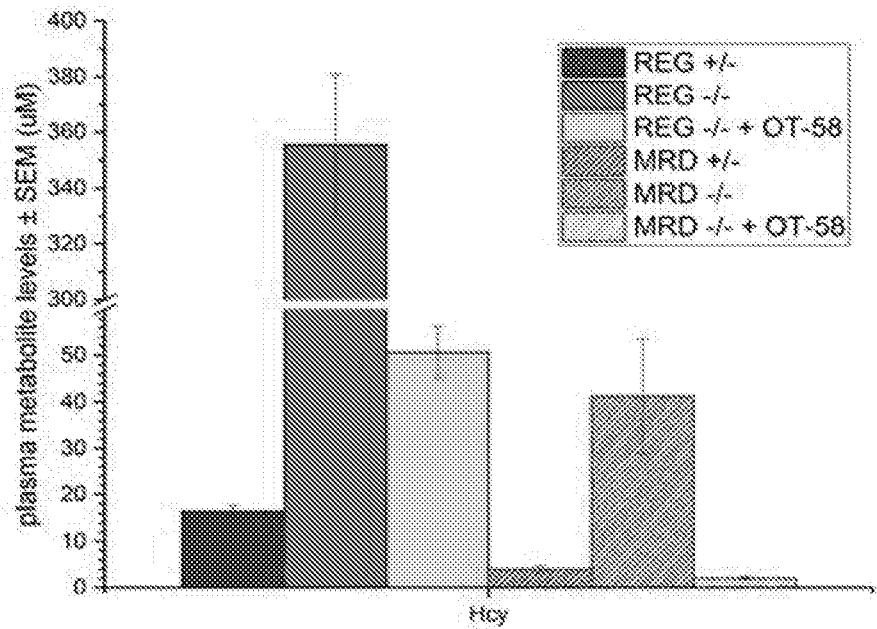


FIG. 20B

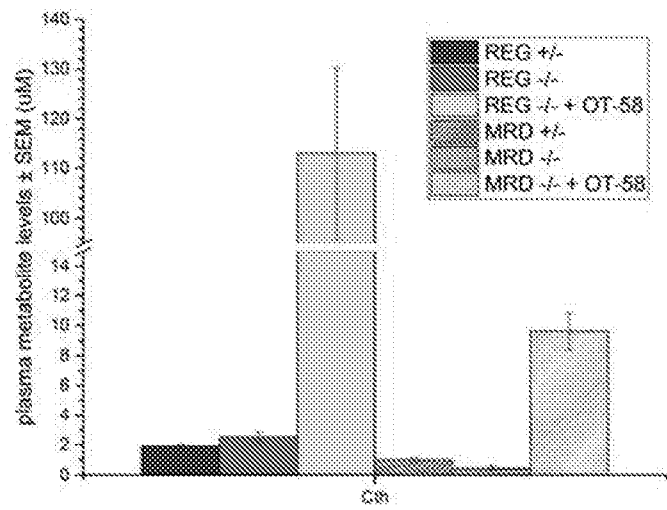


FIG. 20C

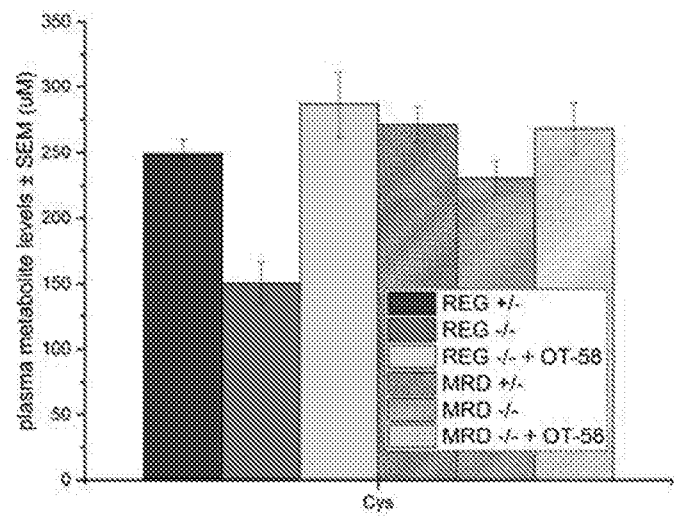


FIG. 20D

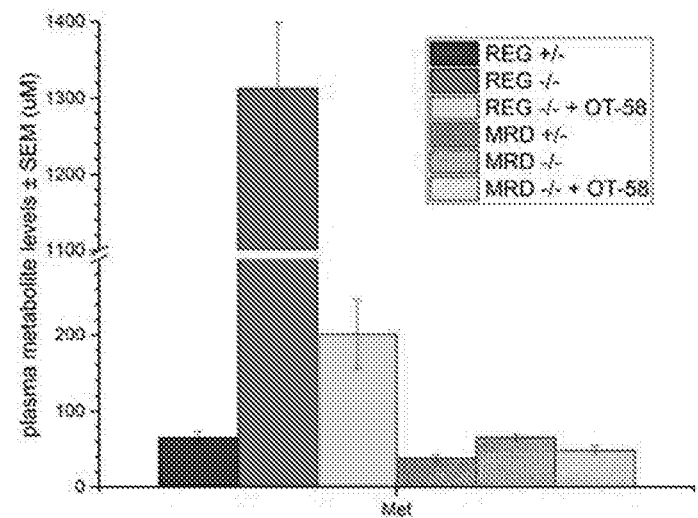


FIG. 21A

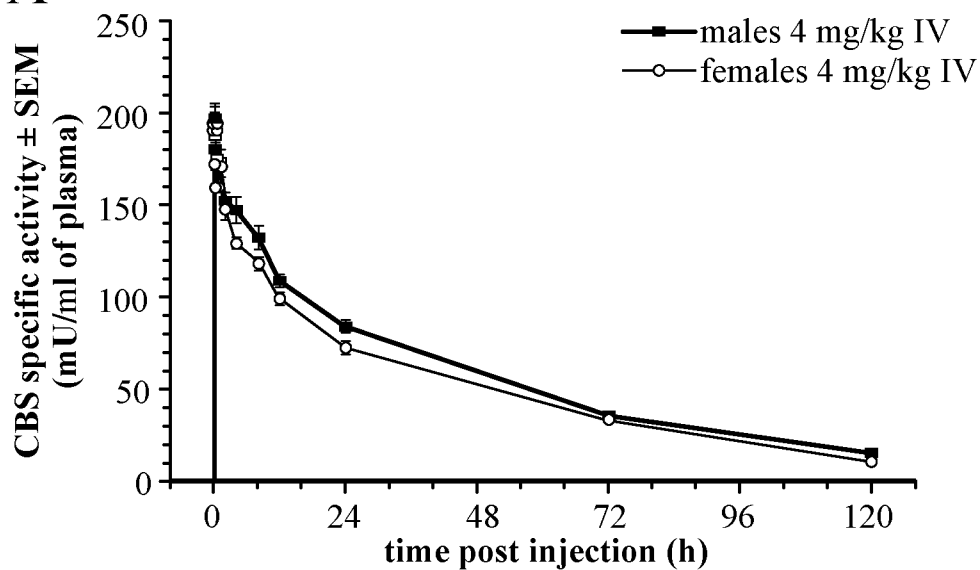


FIG. 21B

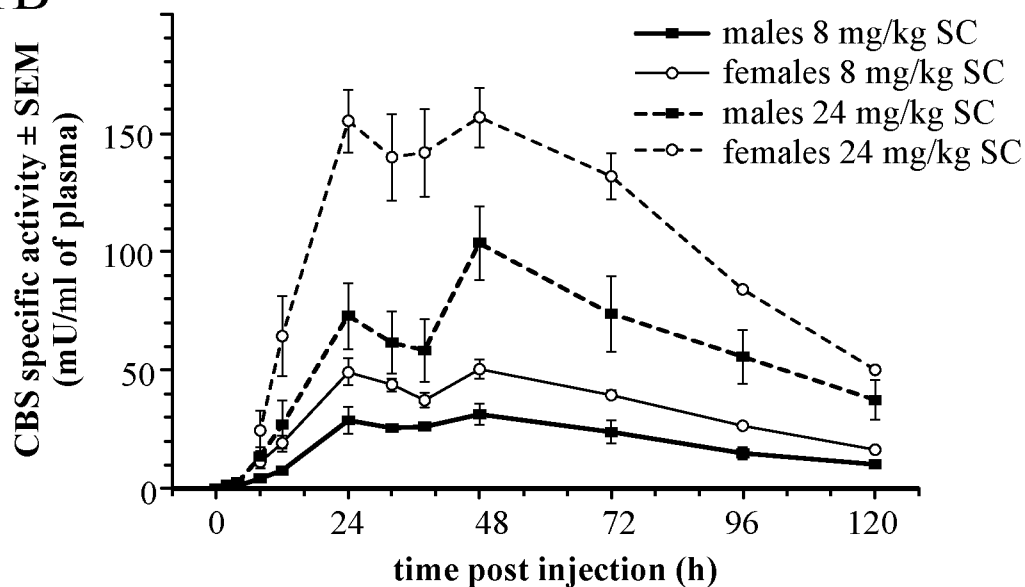


FIG. 21C

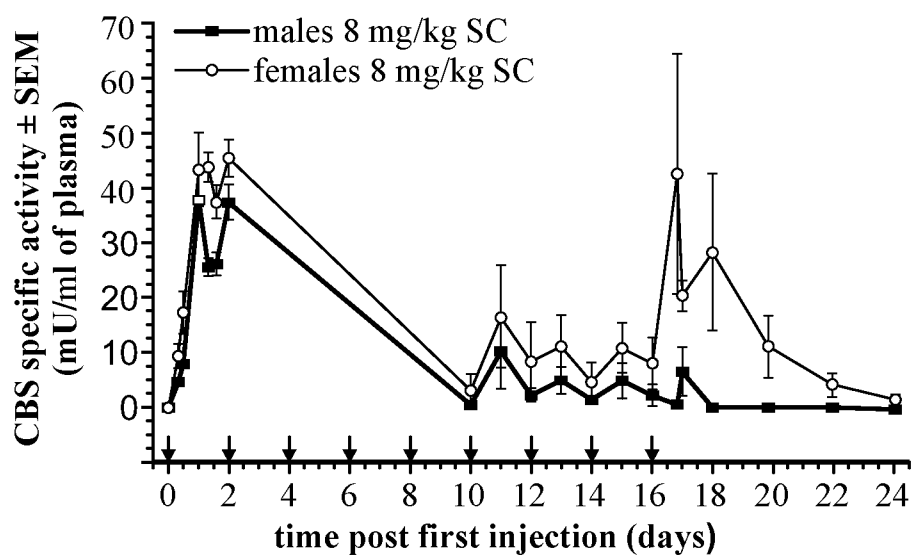


FIG. 22A

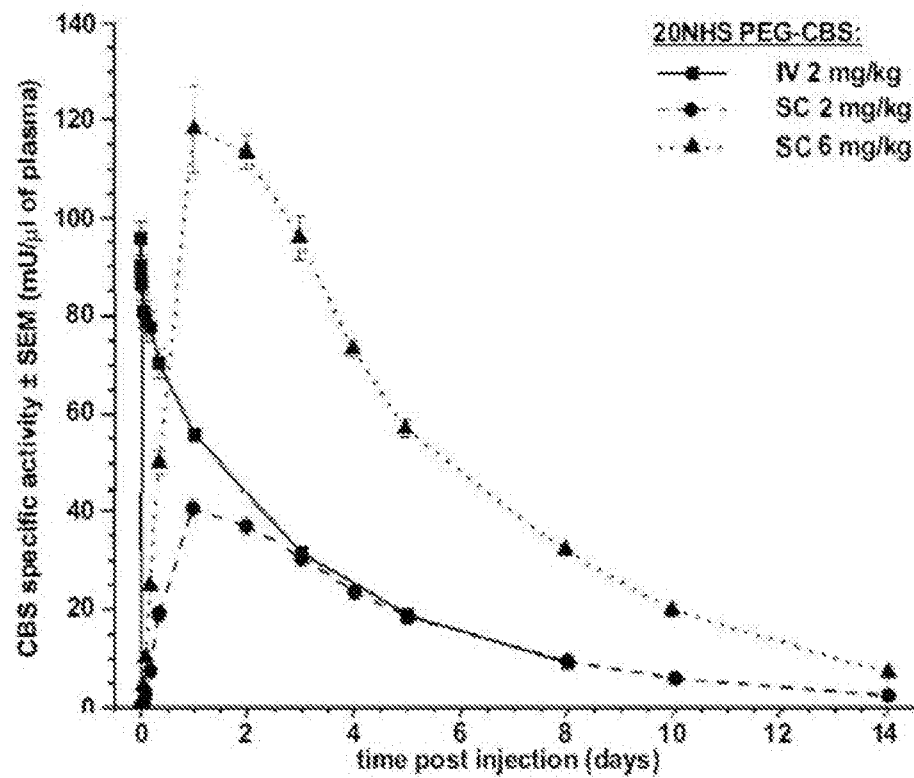


FIG. 22B

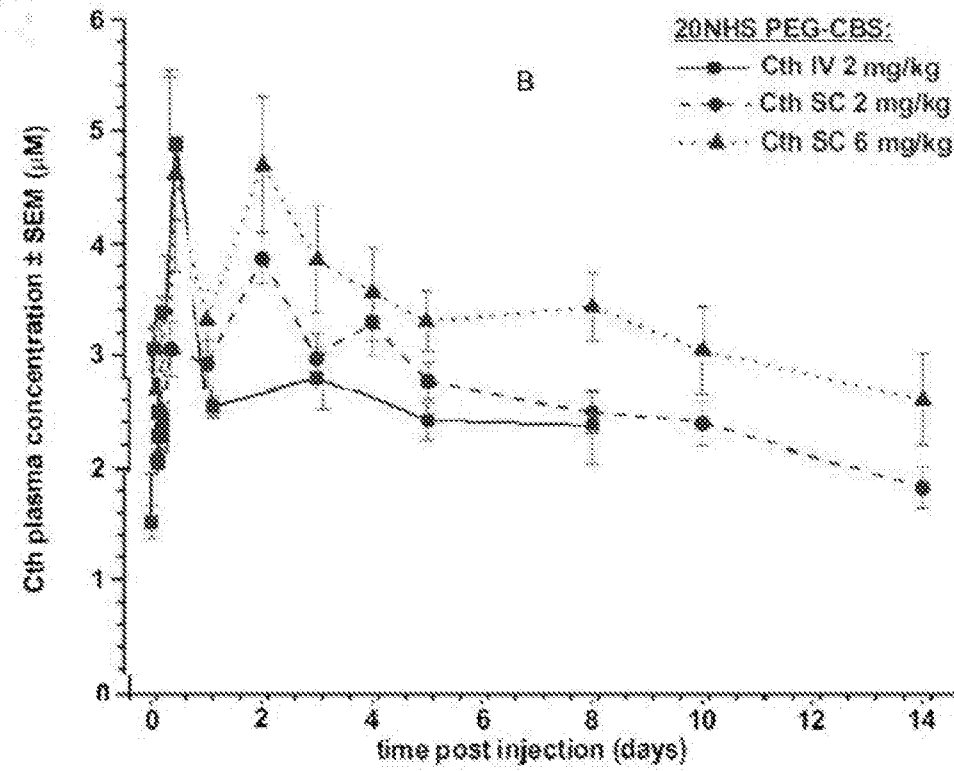


FIG. 23A

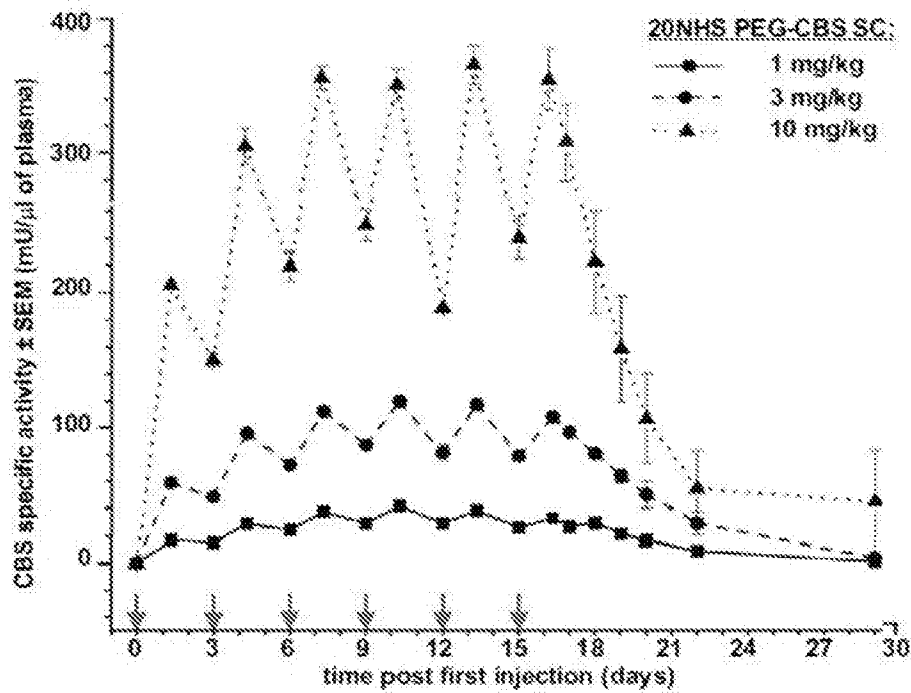


FIG. 23B

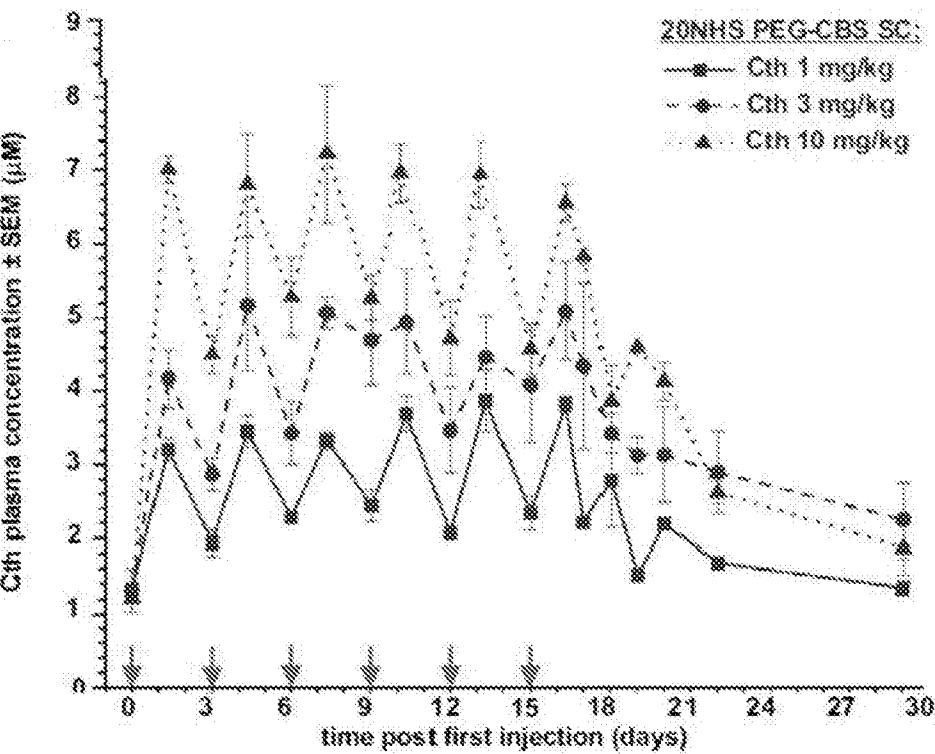


FIG. 24A

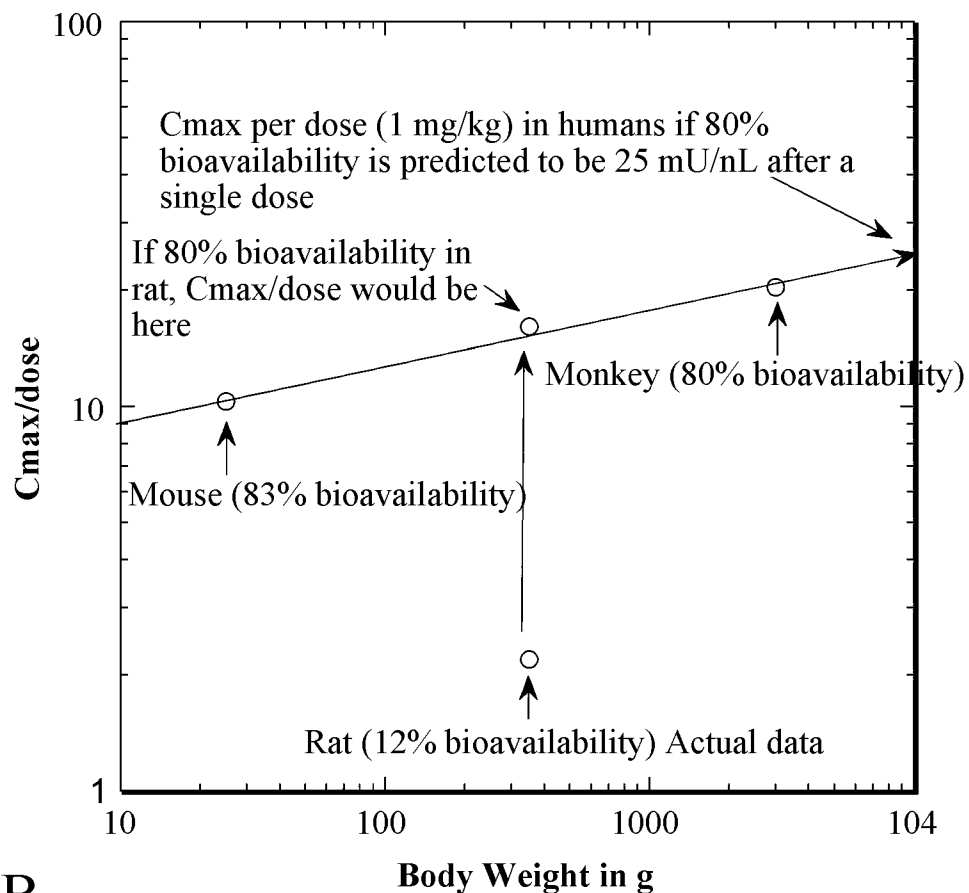


FIG. 24B

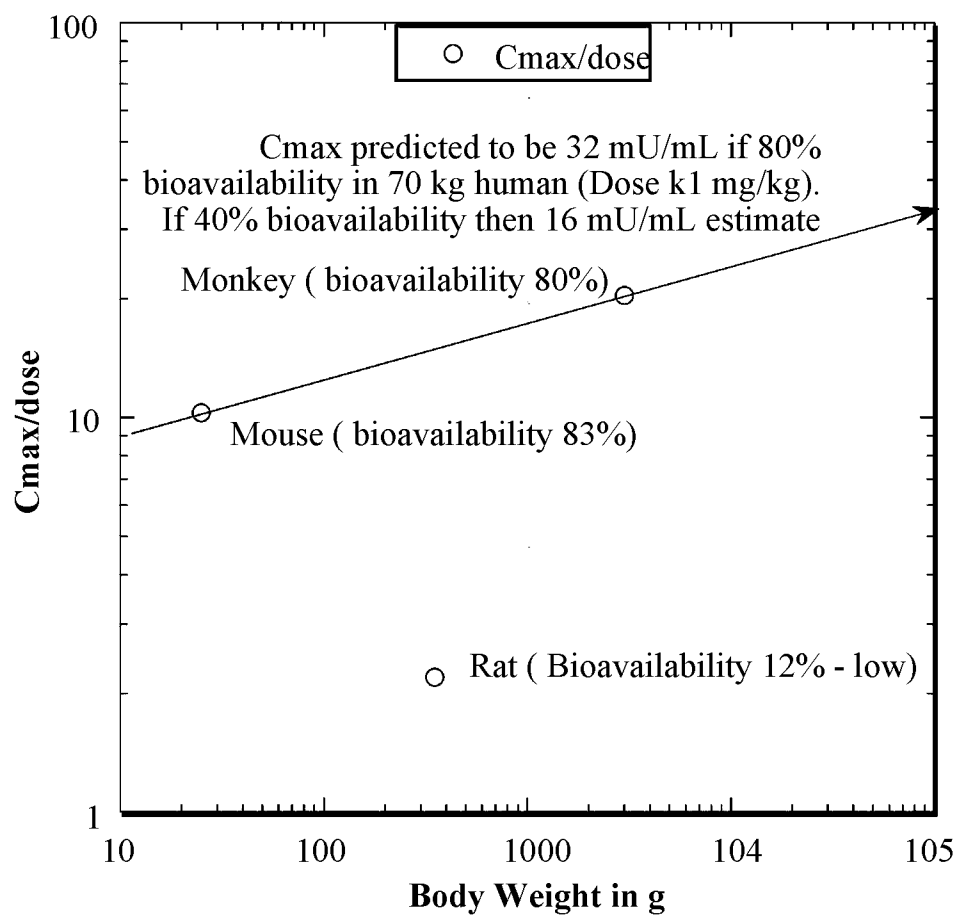


FIG. 24C

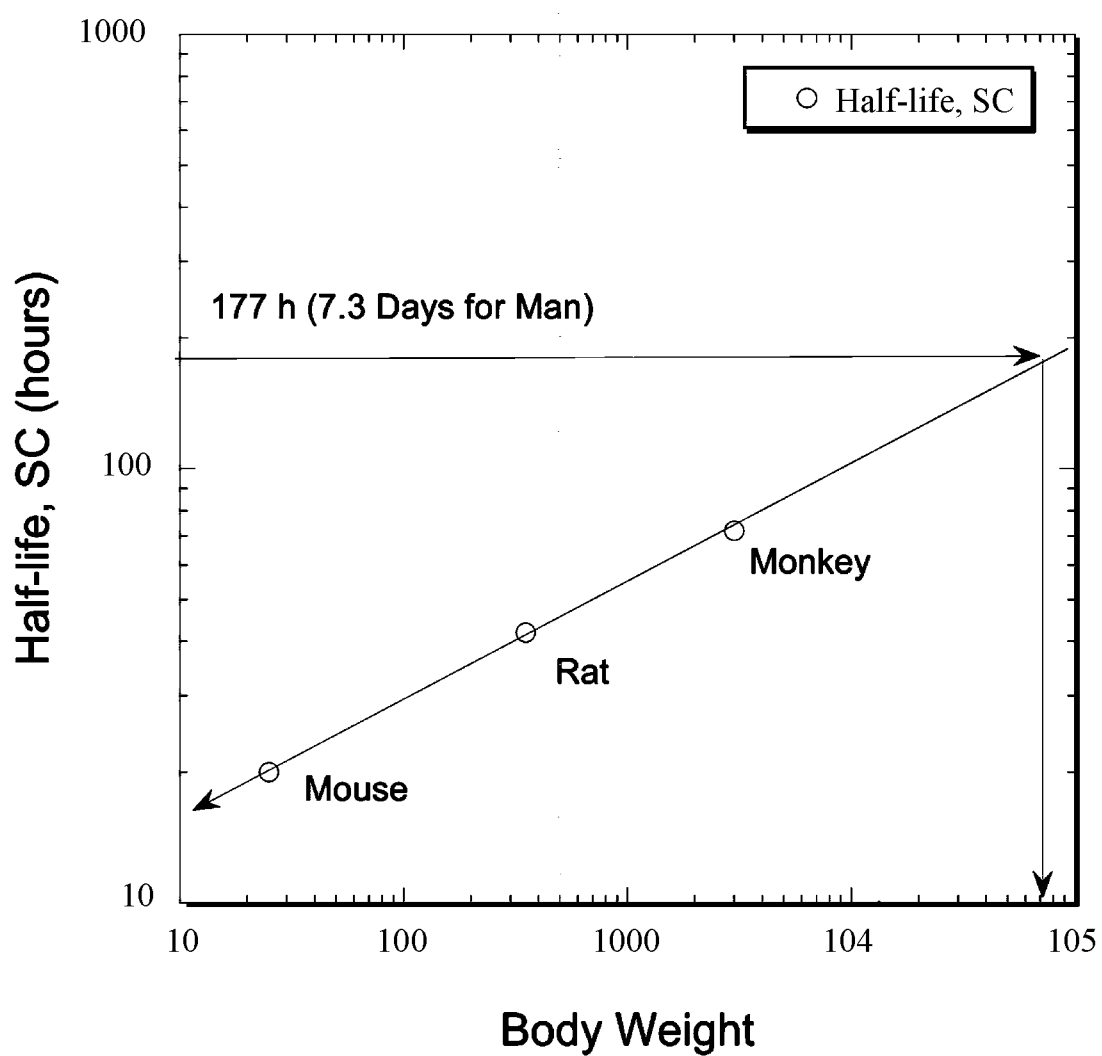
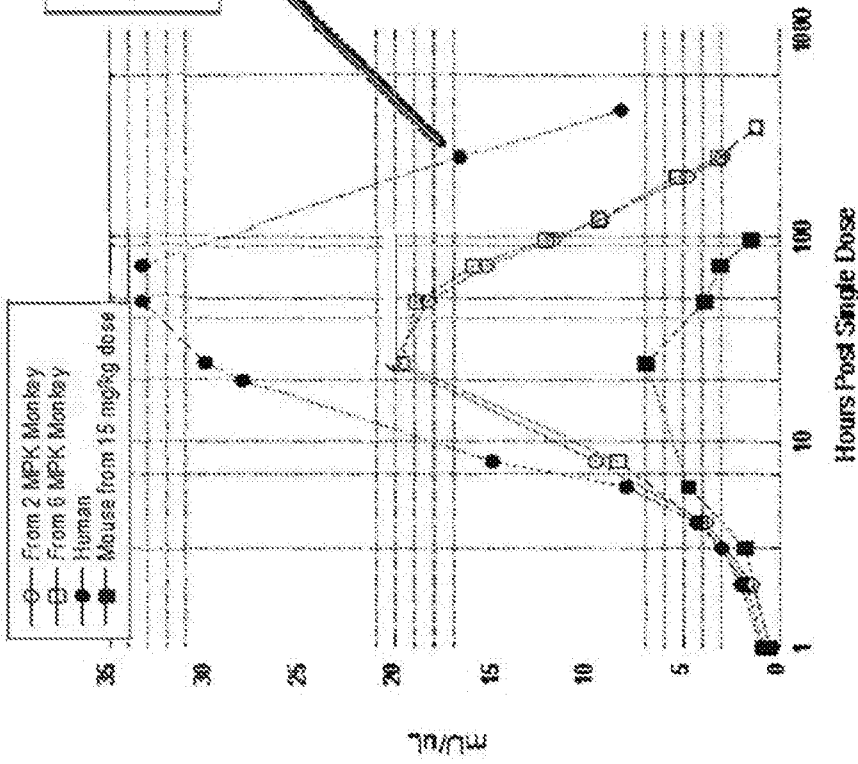


FIG. 24D



Created the human curve with T_{max} 48-72 h,
C_{max} is 34 mU/μL at a single dose of 1 mg/kg
Half-life is 7.3 days :

FIG. 24E

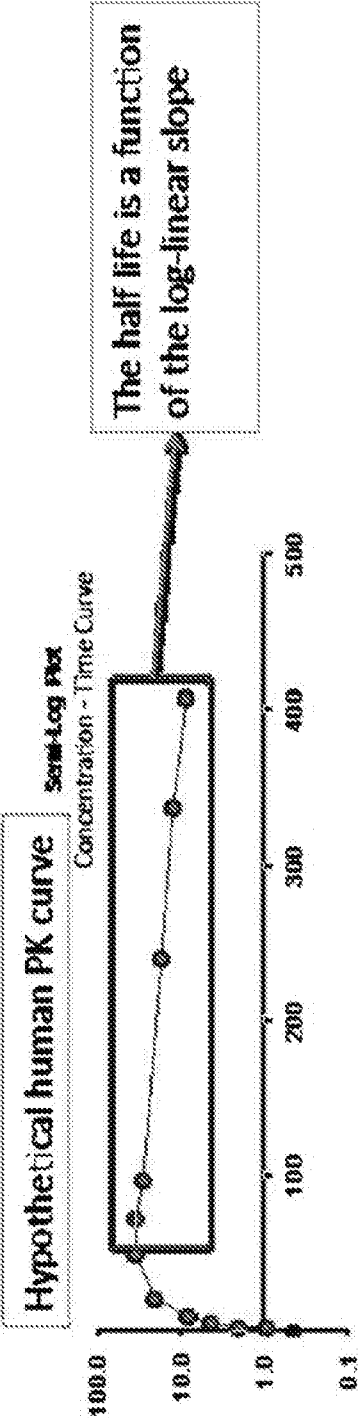


FIG. 24F

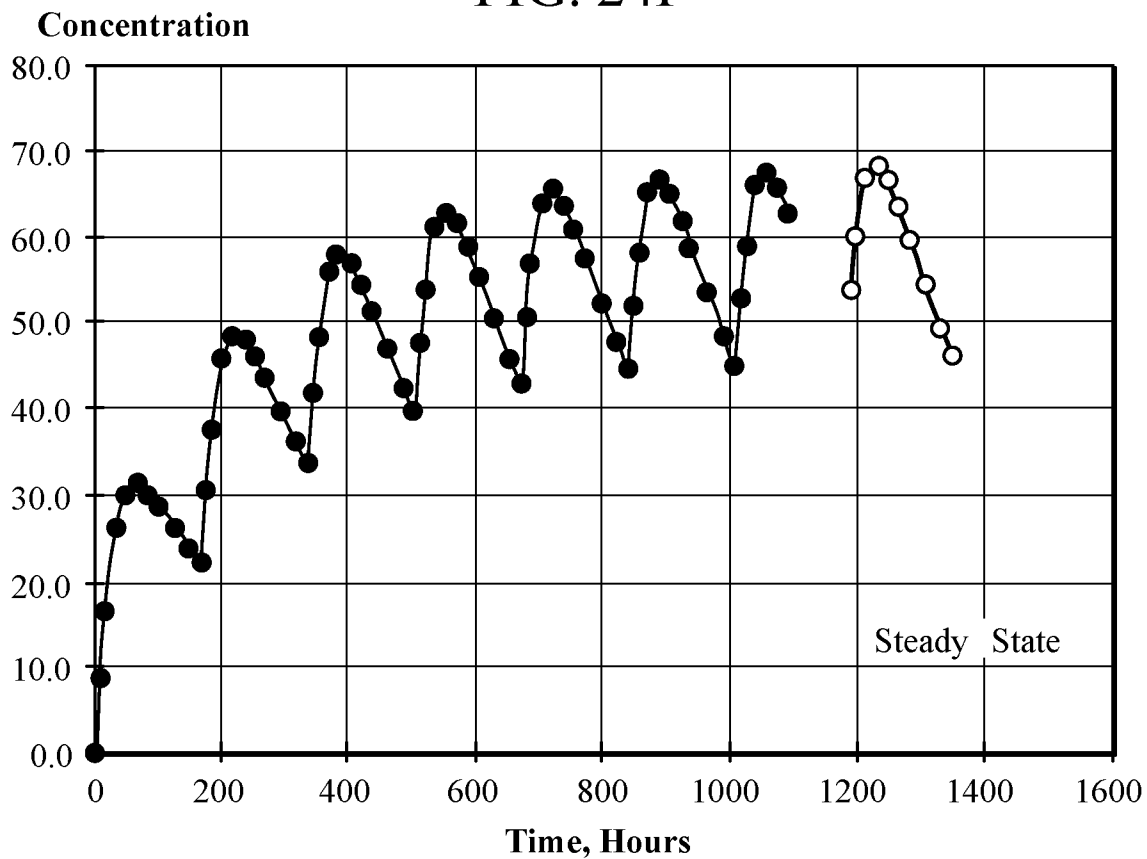


FIG. 24G

