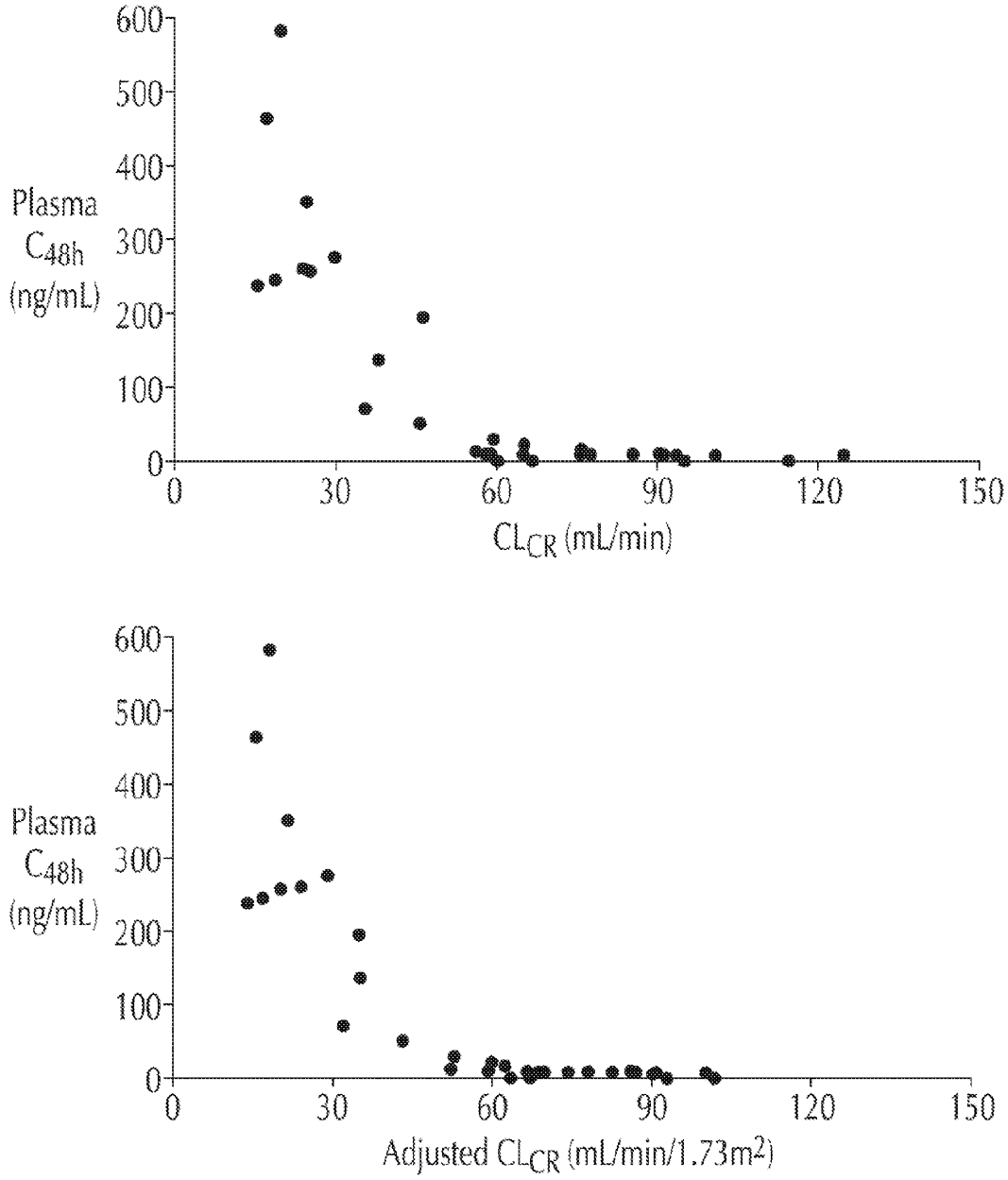

Plasma C_{48h} (ng/mL)
600
500
400
300
200
100
0
0
30
60
90
120
150
CL_{CR} (mL/min)
Plasma C_{48h} (ng/mL)
600
500
400
300
200
100
0
0
30
60
90
120
150
Adjusted CL_{CR} (mL/min/1.73m^2)

FIG. 1A

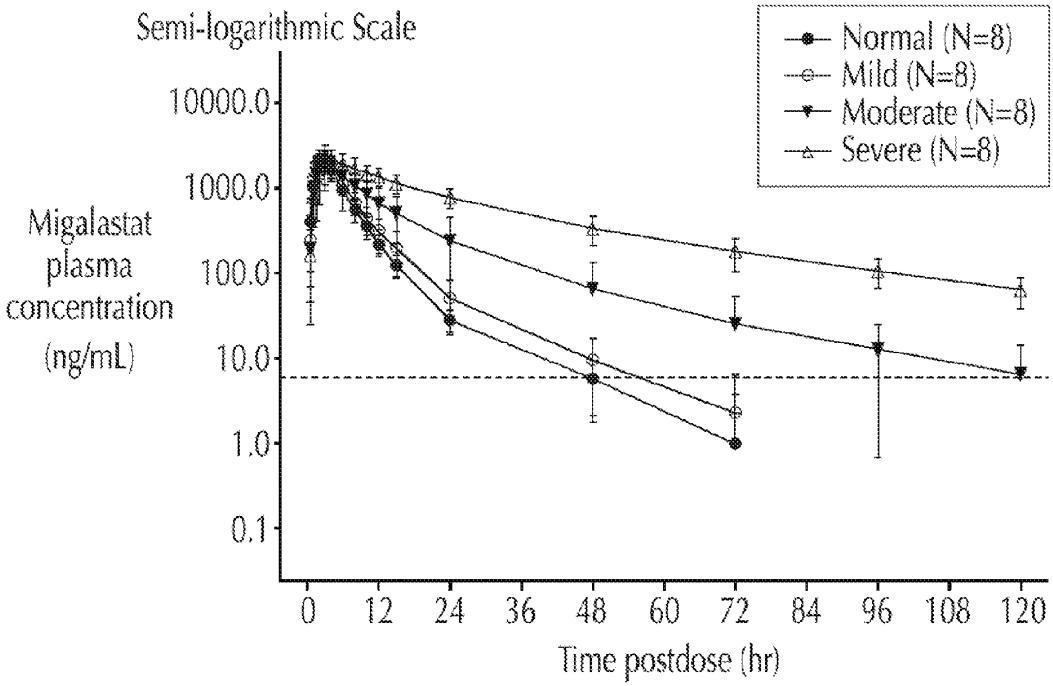

Semi-logarithmic Scale
Normal (N=8)
Mild (N=8)
Moderate (N=8)
Severe (N=8)
Migalastat plasma concentration (ng/mL)
10000.0
1000.0
100.0
10.0
1.0
0.1
0
12
24
36
48
60
72
84
96
108
120
Time postdose (hr)

FIG. 1B

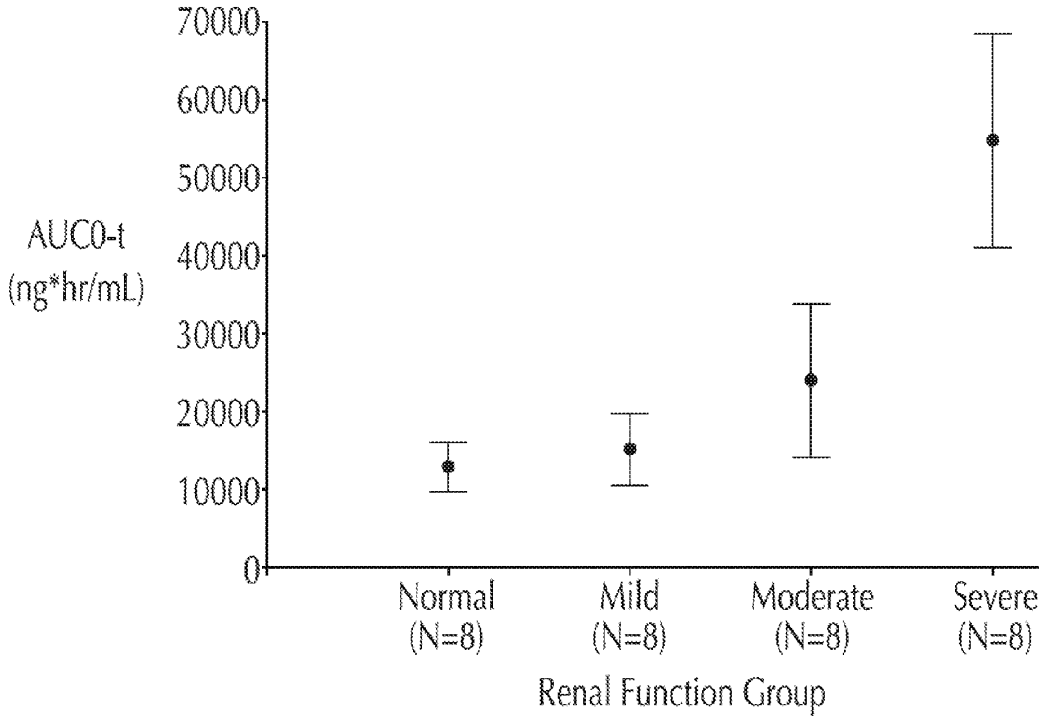

AUC0-t (ng*hr/mL)
70000
60000
50000
40000
30000
20000
10000
0
Normal (N=8)
Mild (N=8)
Moderate (N=8)
Severe (N=8)
Renal Function Group

FIG. 1C

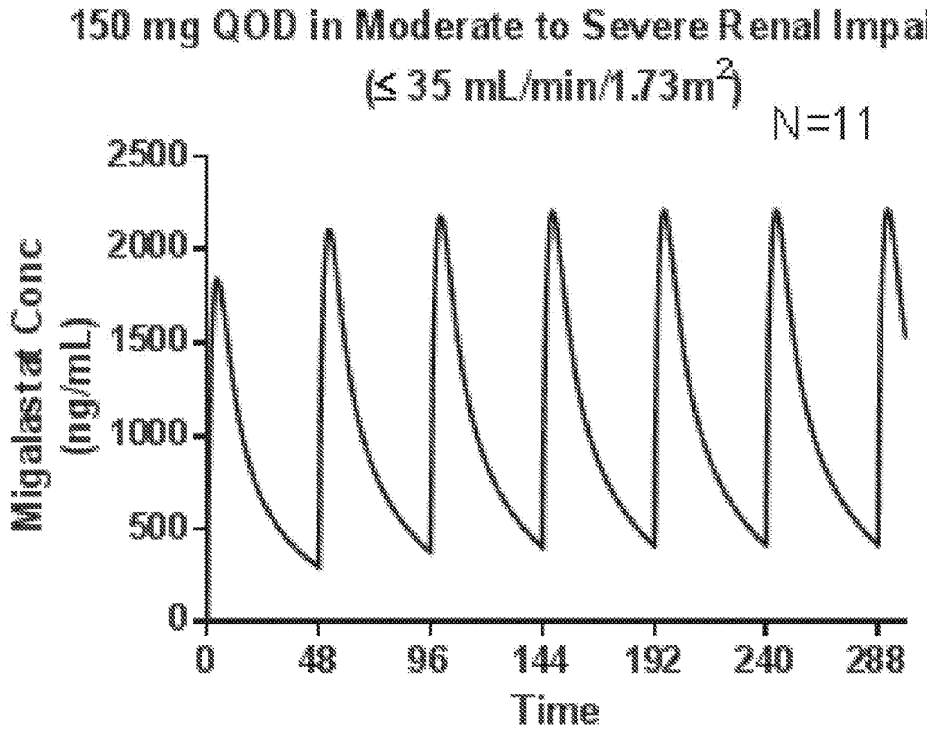

150 mg QOD in Moderate to Severe Renal Impairment
(≤ 35 mL/min/1.73m²)
N=11
Migalastat Conc (ng/mL)
2500
2000
1500
1000
500
0
0
48
96
144
192
240
288
Time

FIG. 2A

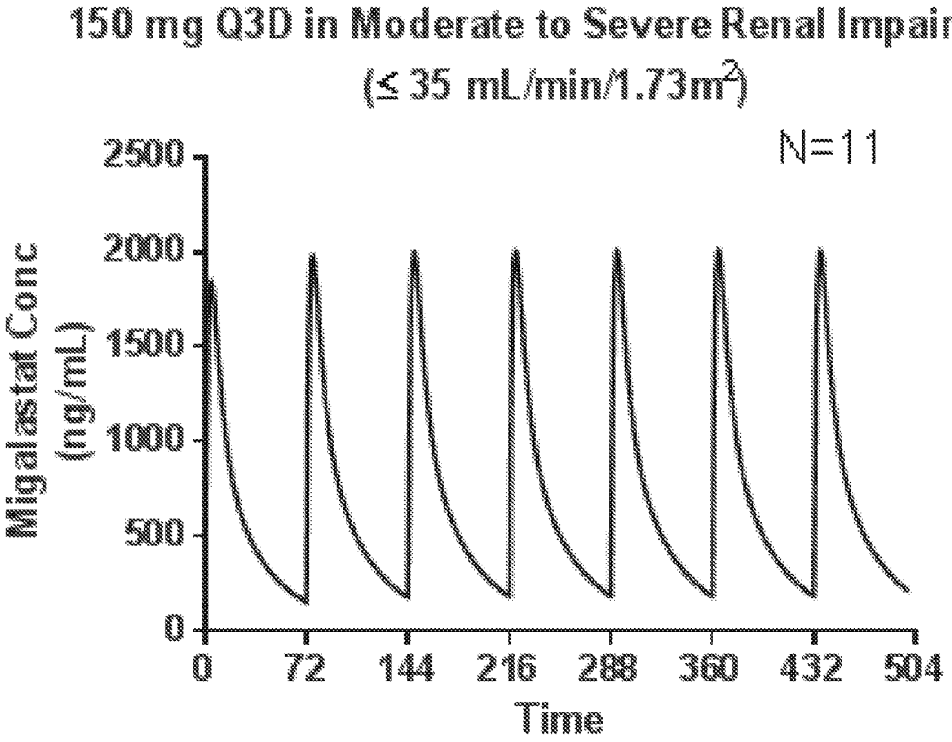

150 mg Q3D in Moderate to Severe Renal Impaiment
(≤ 35 mL/min/1.73m²)
N=11
Migalastat Conc (ng/mL)
2500
2000
1500
1000
500
0
0
72
144
216
288
360
432
504
Time

FIG. 2B

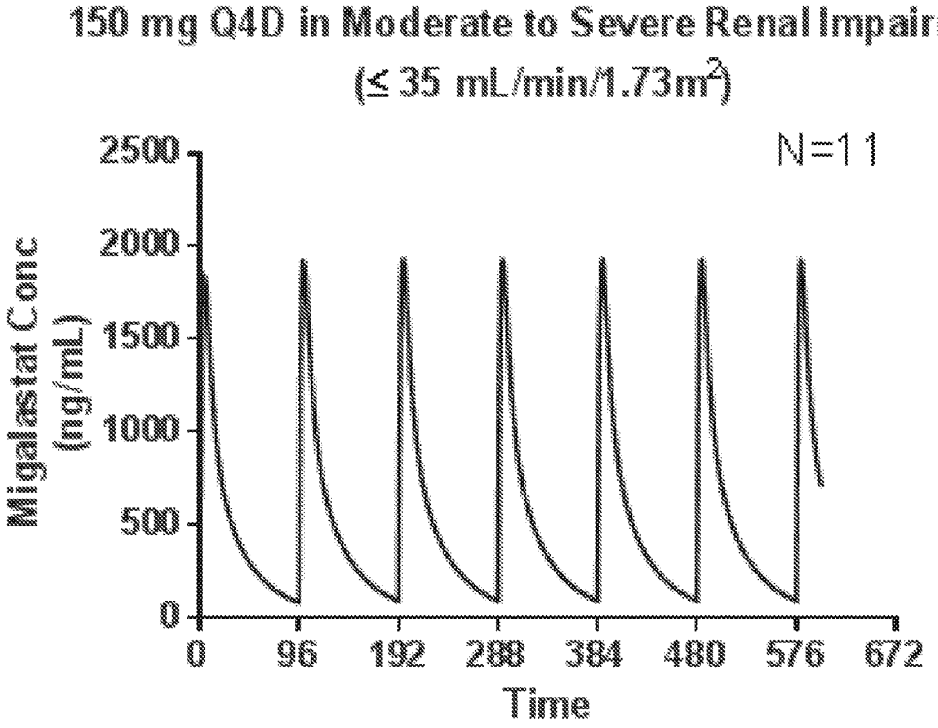

150 mg Q4D in Moderate to Severe Renal Impairment
(≤ 35 mL/min/1.73m2)
N=11
Migalastat Conc (ng/mL)
2500
2000
1500
1000
500
0
0
96
192
288
384
480
576
672
Time

FIG. 2C

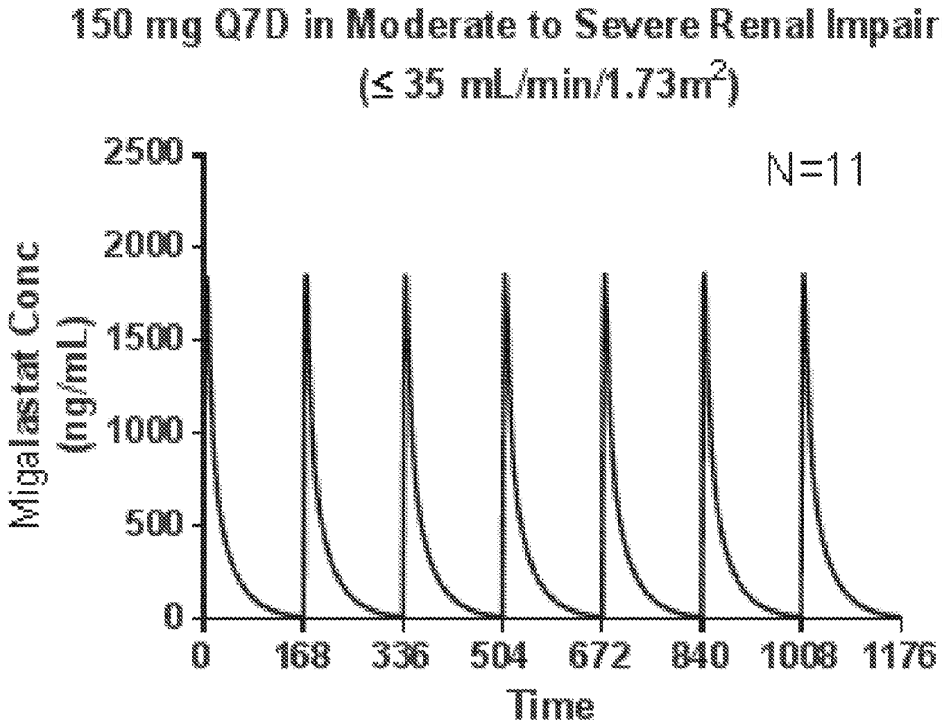

150 mg Q7D in Moderate to Severe Renal Impairment
(≤ 35 mL/min/1.73m2)
N=11
Migalastat Conc (ng/mL)
2500
2000
1500
1000
500
0
0
168
336
504
672
840
1008
1176
Time

FIG. 2D

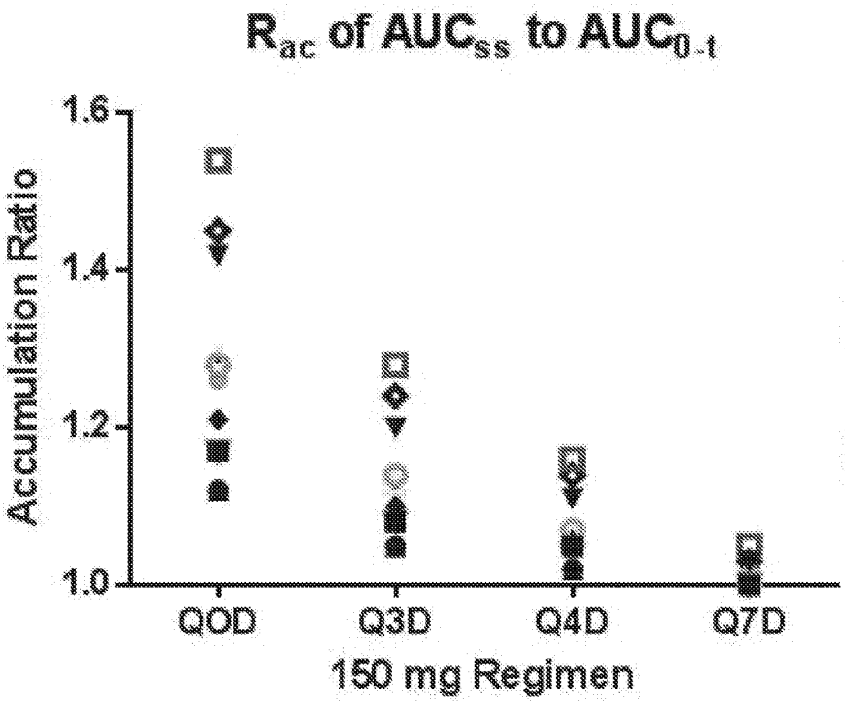

R_{ac} of AUC_{ss} to AUC_{0-t}
Accumulation Ratio
1.6
1.4
1.2
1.0
QOD
Q3D
Q4D
Q7D
150 mg Regimen

FIG. 3A

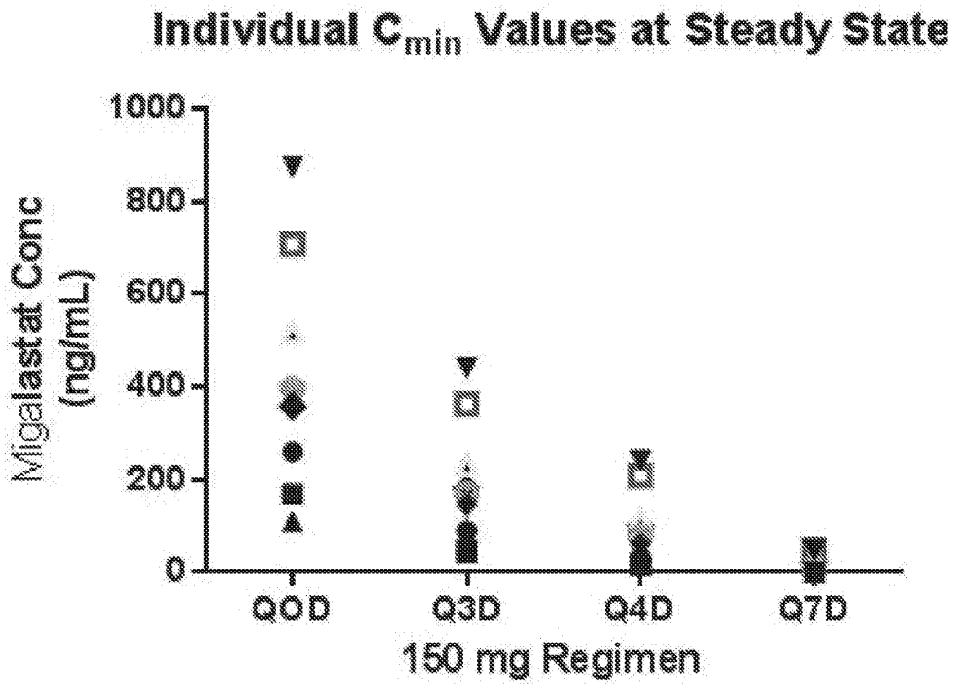

Individual C_{min} Values at Steady State
Migalastat Conc (ng/mL)
1000
800
600
400
200
0
QOD
Q3D
Q4D
Q7D
150 mg Regimen

FIG. 3B

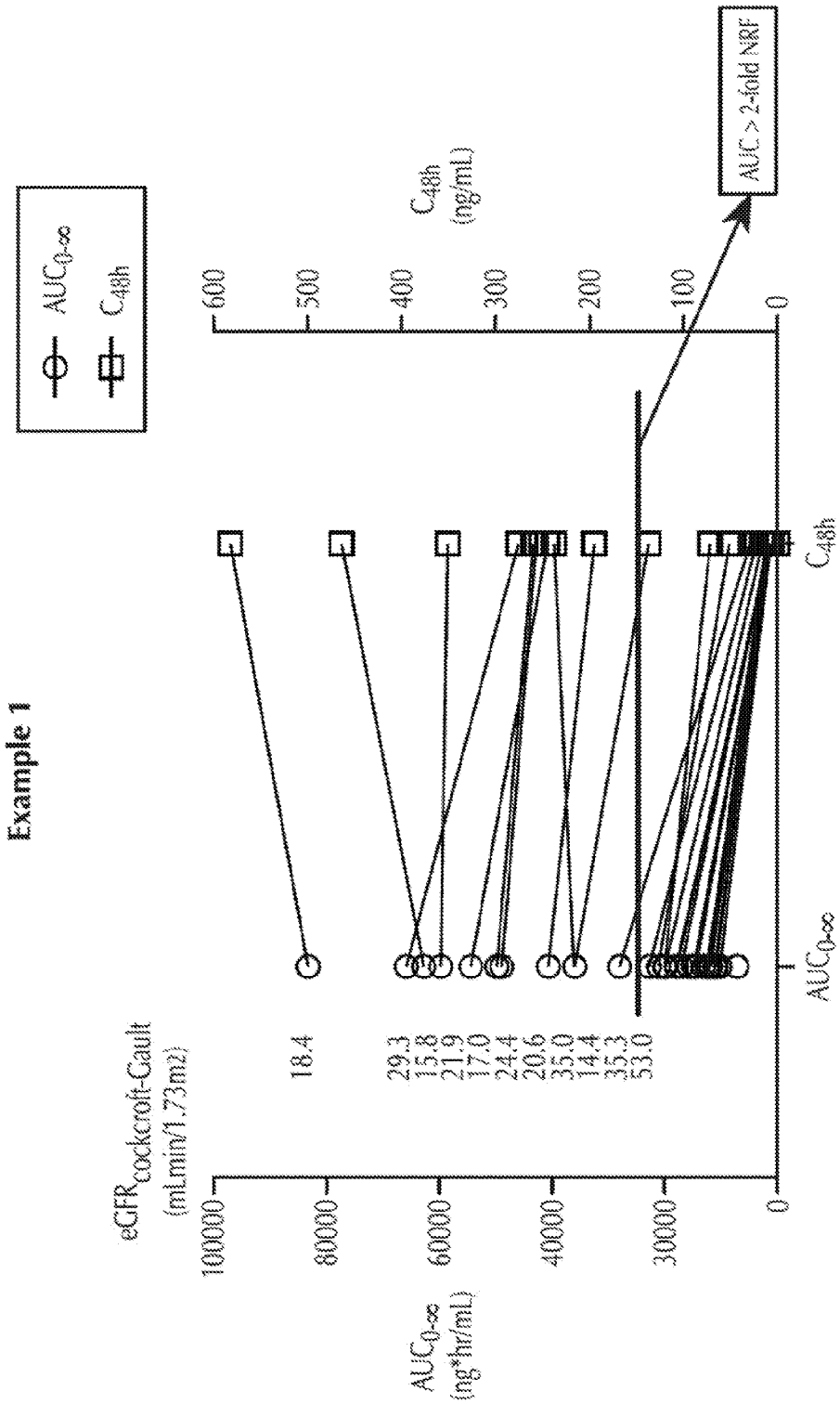

Example 1
AUC0-∞
C48h
C48h (ng/mL)
600
500
400
300
200
100
0
AUC > 2-fold NRF
C48h
AUC0-∞
eGFRCockcroft-Gault (mL/min/1.73m2)
18.4
29.3
15.8
21.9
17.0
24.4
20.6
35.0
14.4
35.3
53.0
100000
80000
60000
40000
30000
0
AUC0-∞ (ng*hr/mL)

FIG. 4

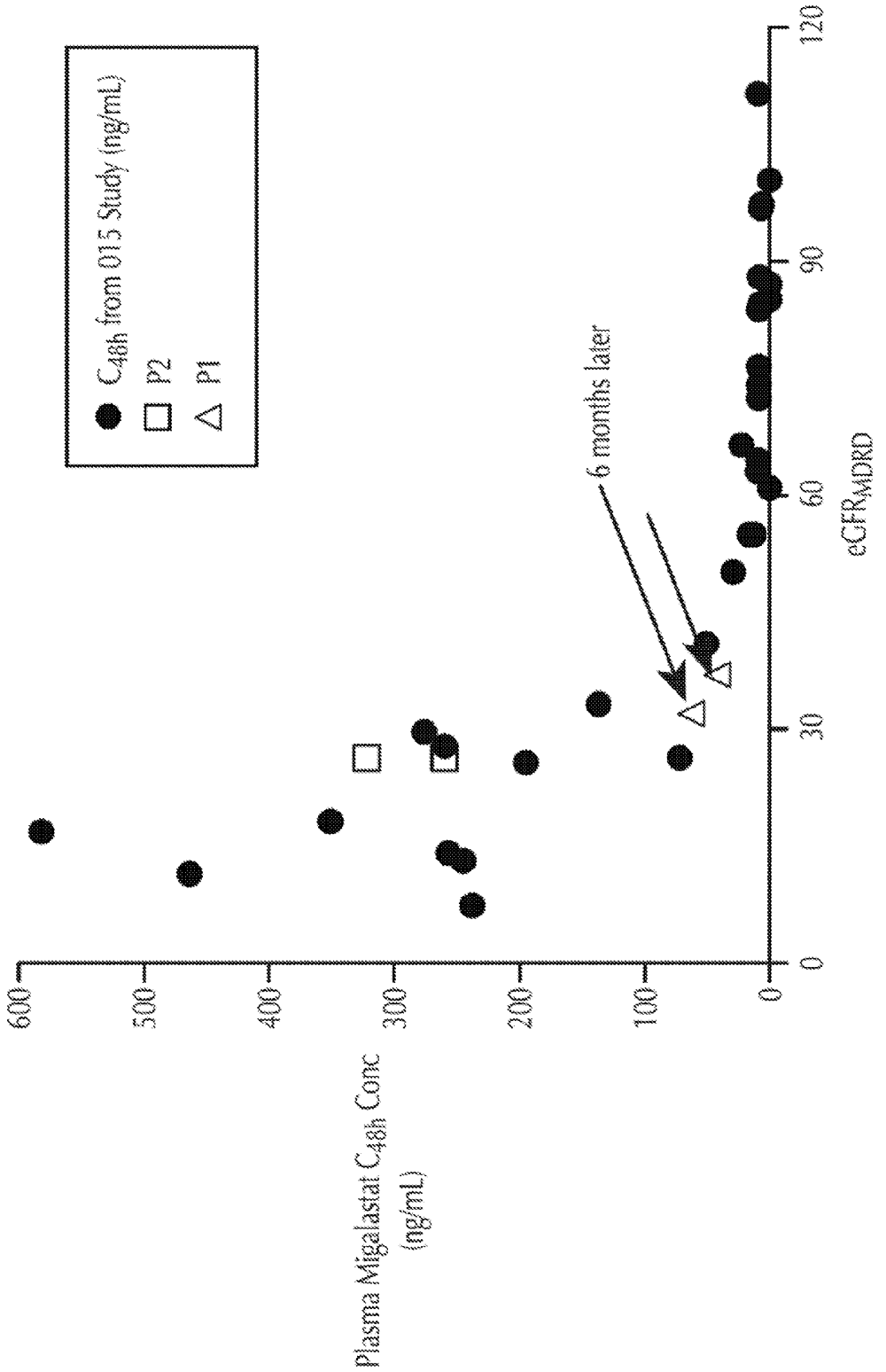

C_{48h} from 015 Study (ng/mL)
P2
P1
6 months later
Plasma Miglalastat C_{48h} Conc (ng/mL)
600
500
400
300
200
100
0
0
30
60
90
120
eGFR$_{MDRD}$

FIG. 5

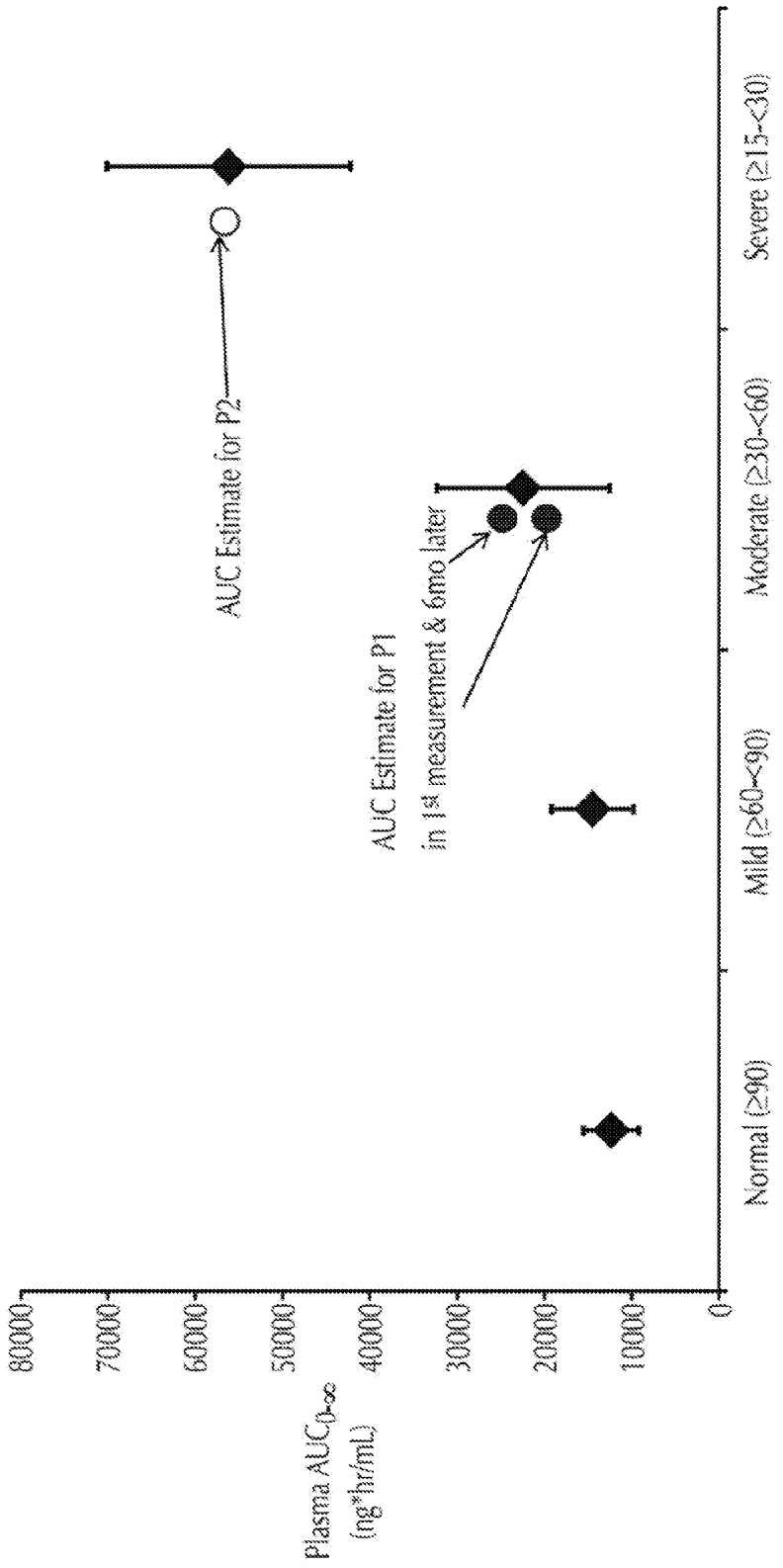

AUC Estimate for P2
AUC Estimate for P1
in 1st measurement & 6mo later
Plasma AUC0-∞
(ng*hr/mL)
80000
70000
60000
50000
40000
30000
20000
10000
0
Normal (≥90)
Mild (≥60-<90)
Moderate (≥30-<60)
Severe (≥15-<30)
Renal Function Group (range mL/min)

FIG. 6

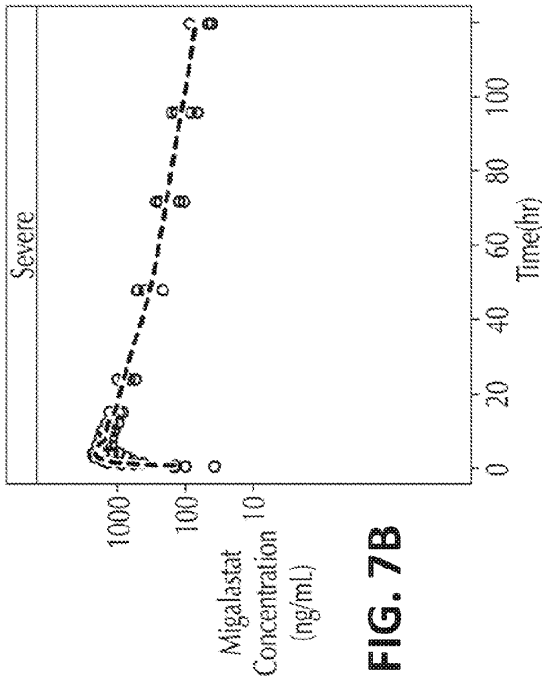


FIG. 7B

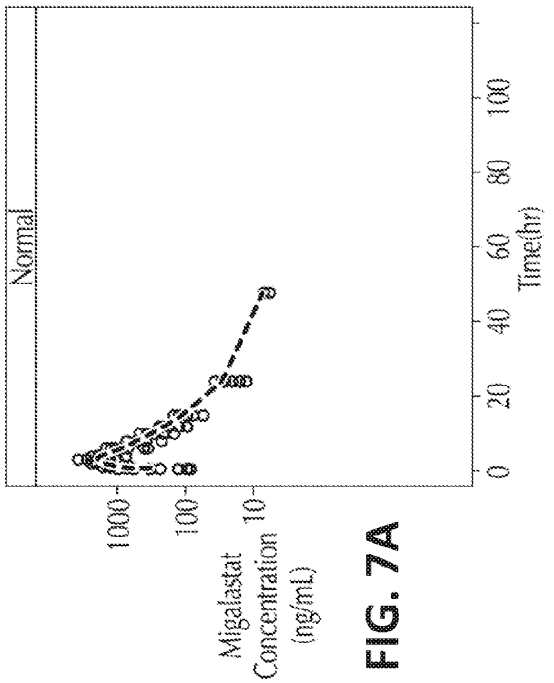


FIG. 7A

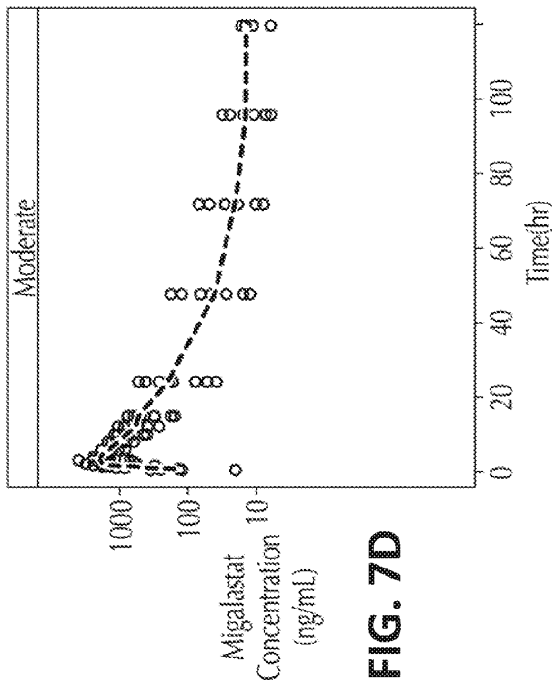


FIG. 7D

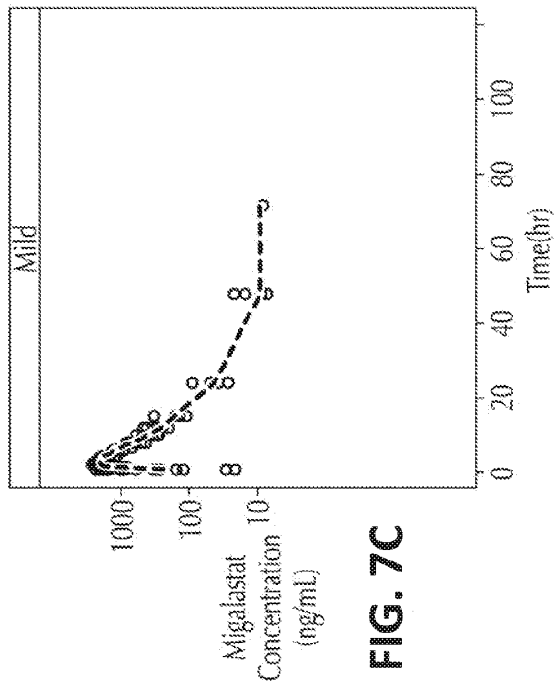


FIG. 7C

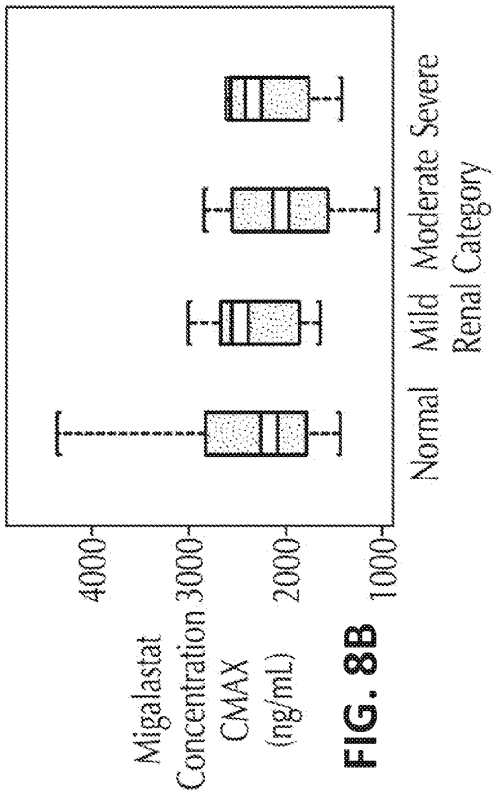


FIG. 8B

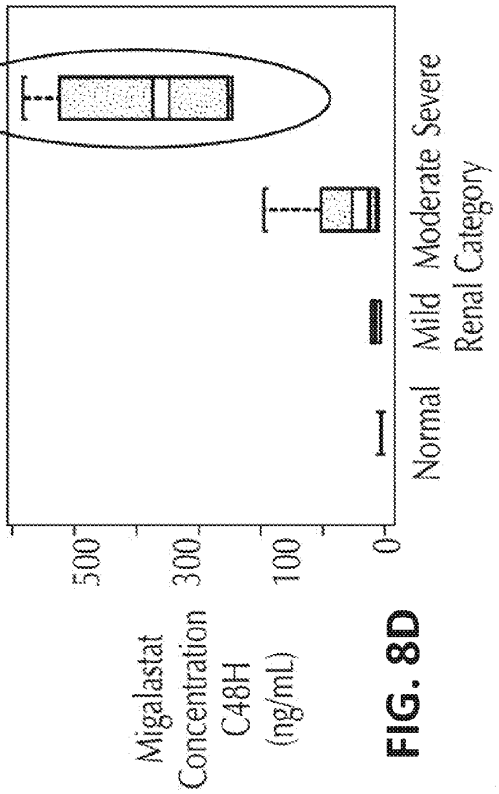


FIG. 8D

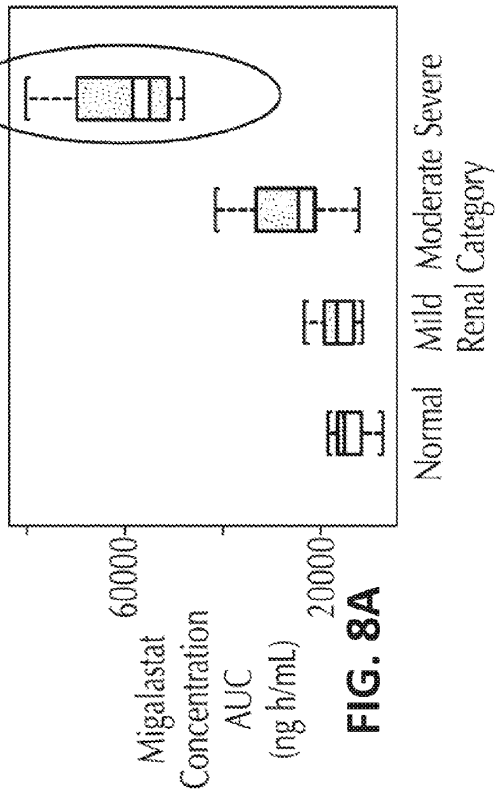


FIG. 8A

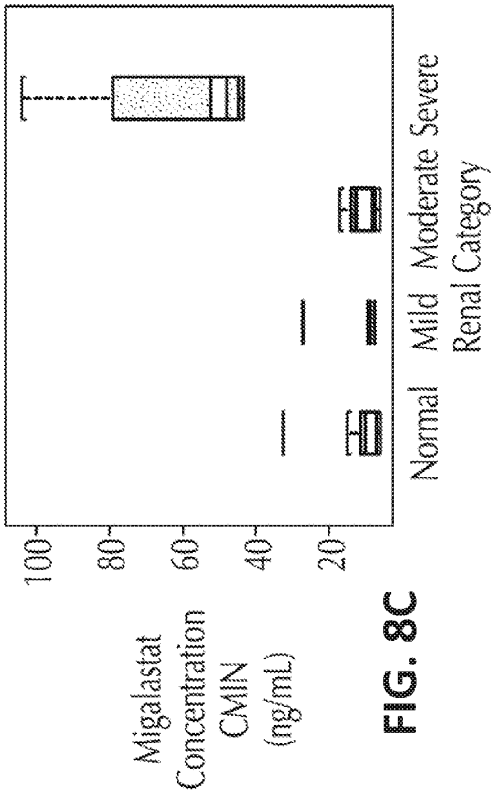


FIG. 8C

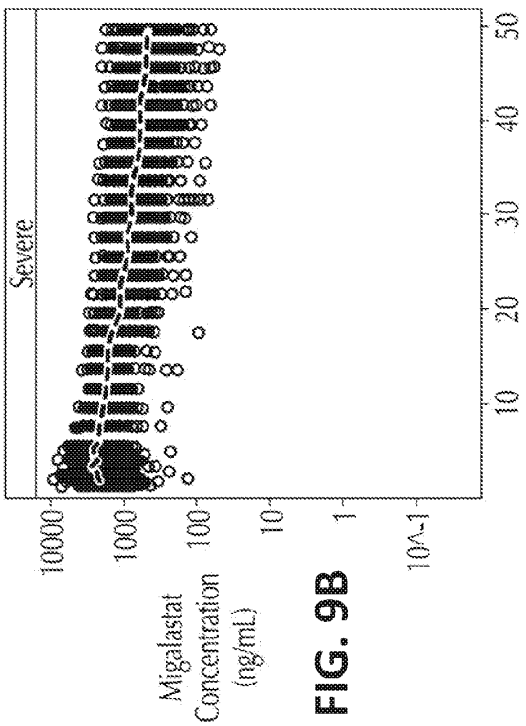


FIG. 9B

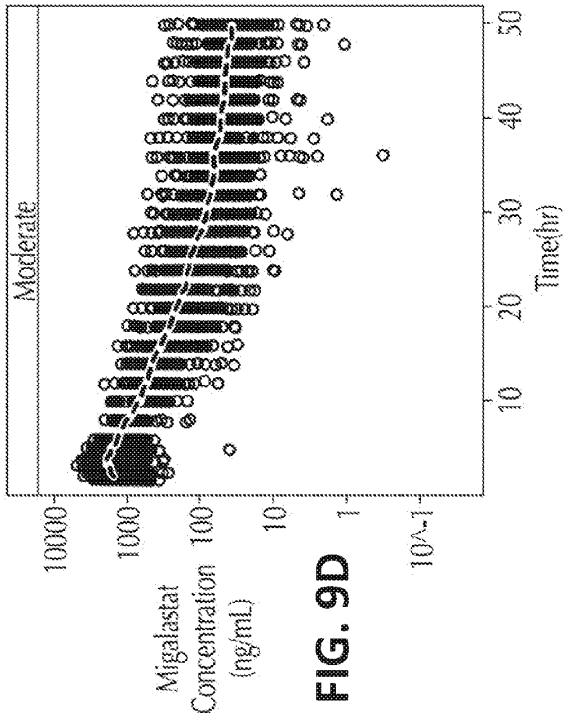


FIG. 9D

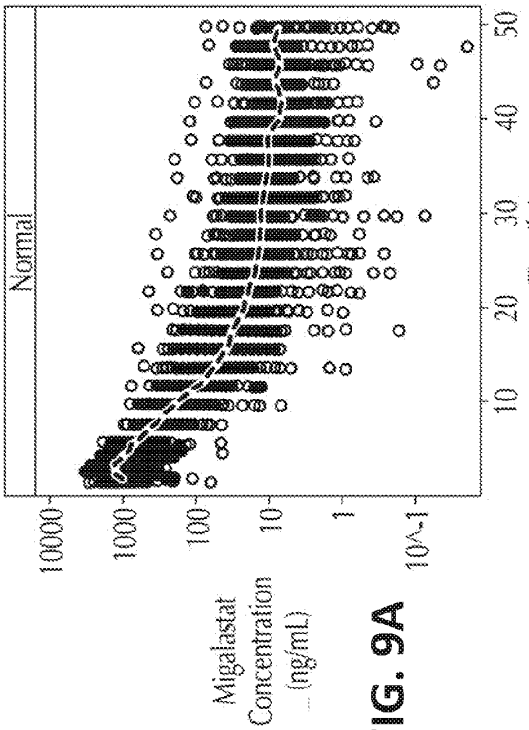


FIG. 9A

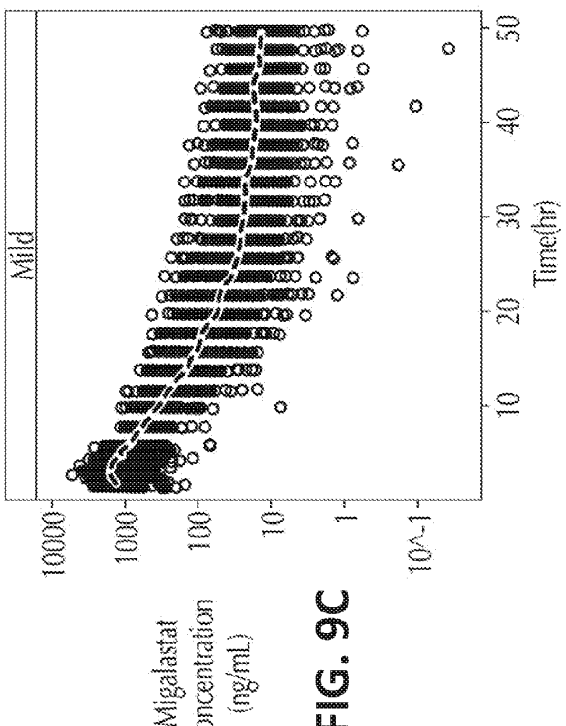


FIG. 9C

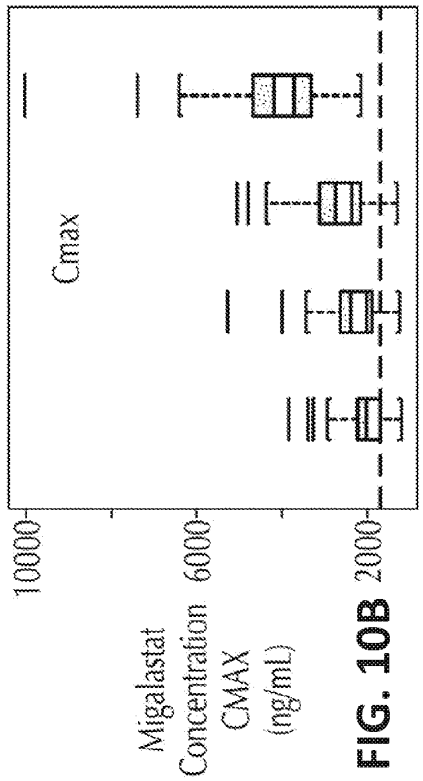


FIG. 10B

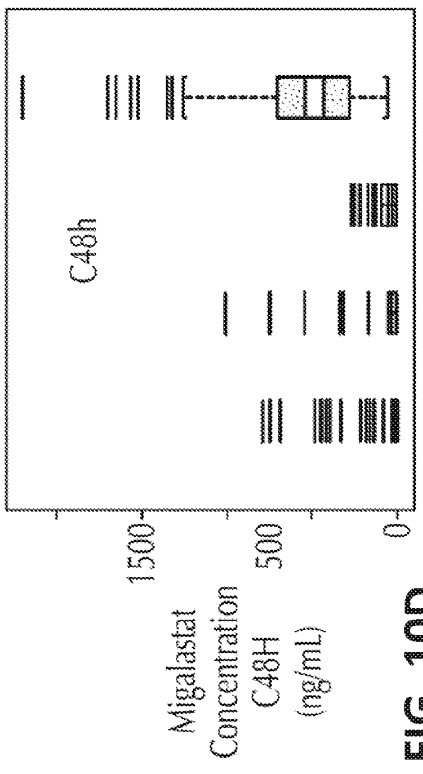


FIG. 10D

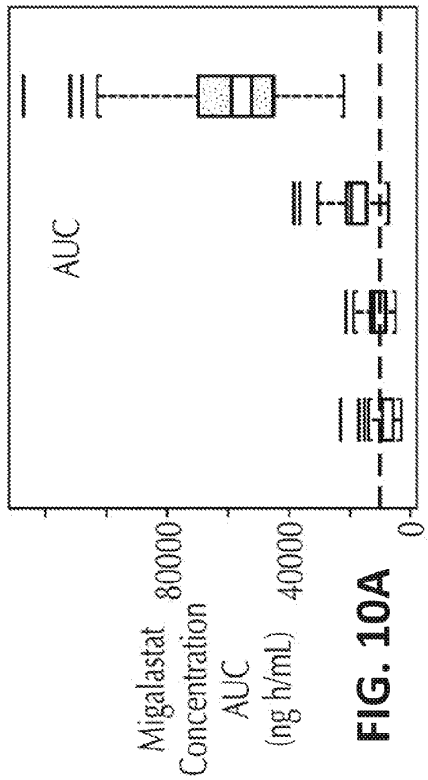


FIG. 10A

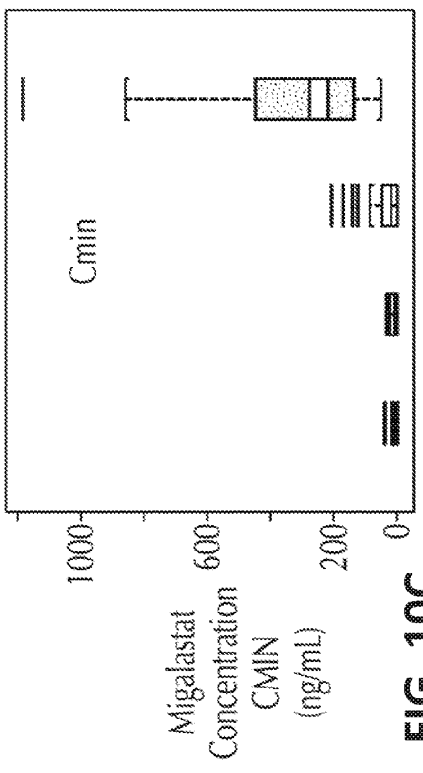


FIG. 10C

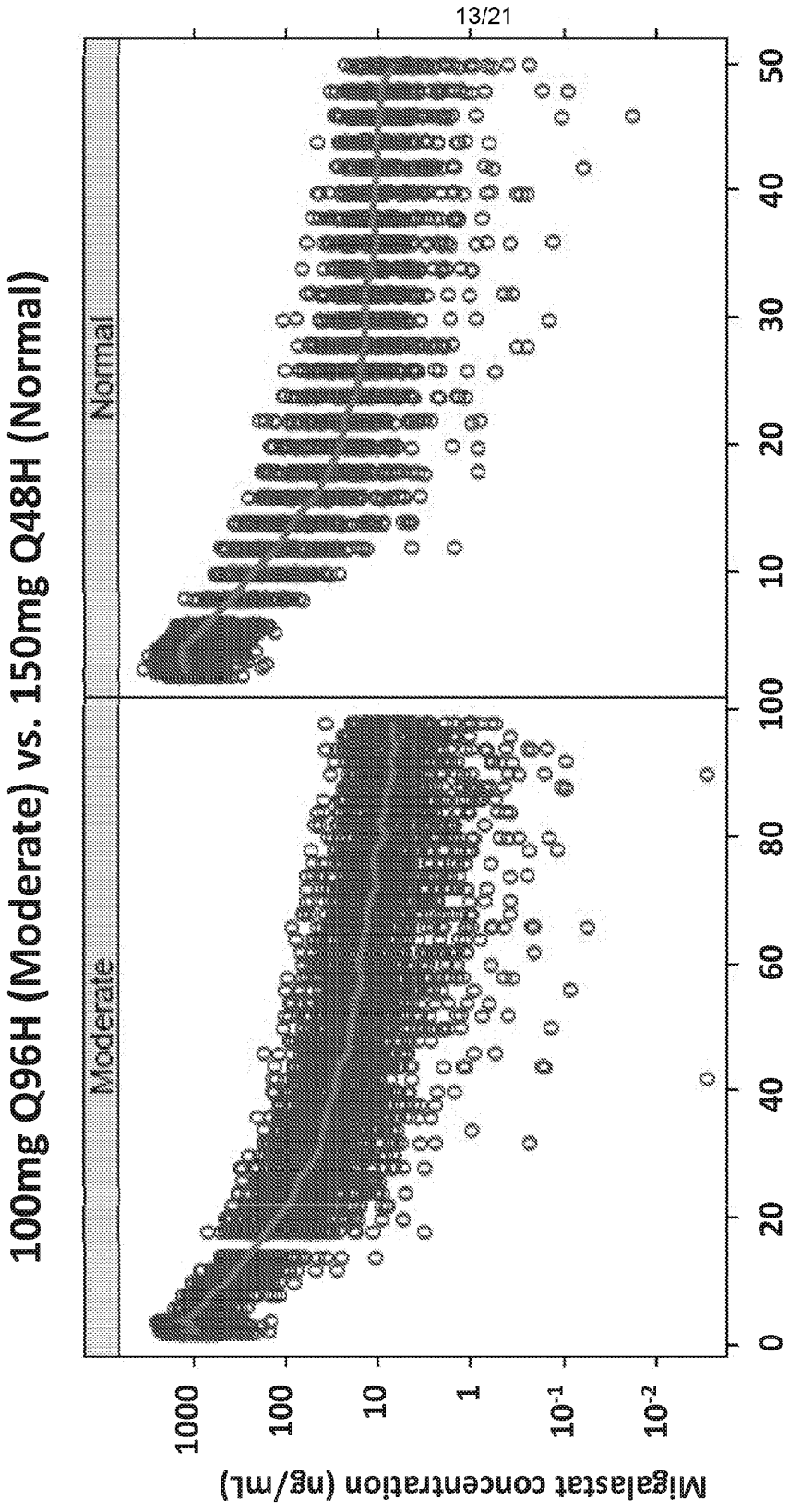


FIG. 11A

FIG.11B

FIG. 12A

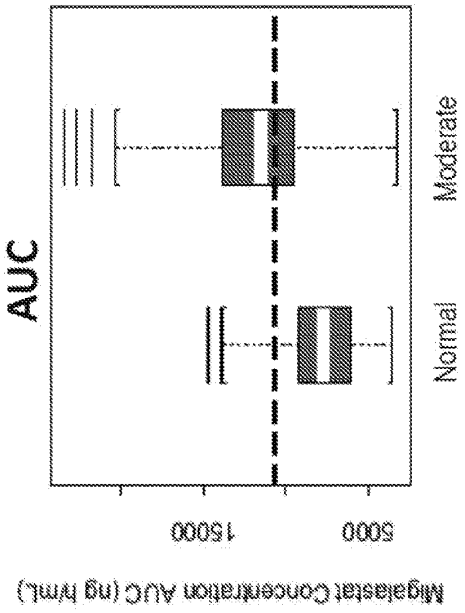


FIG. 12B

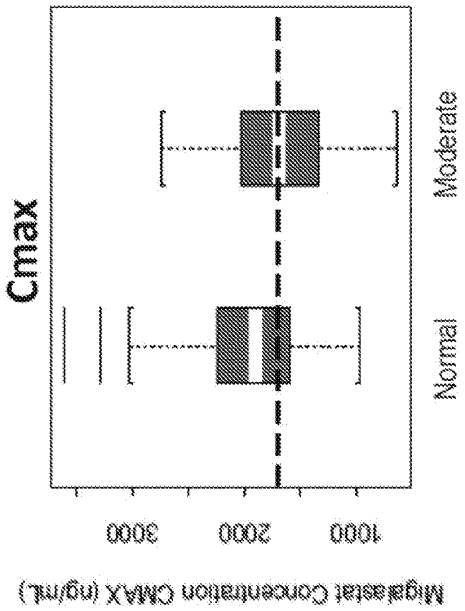


FIG. 12C

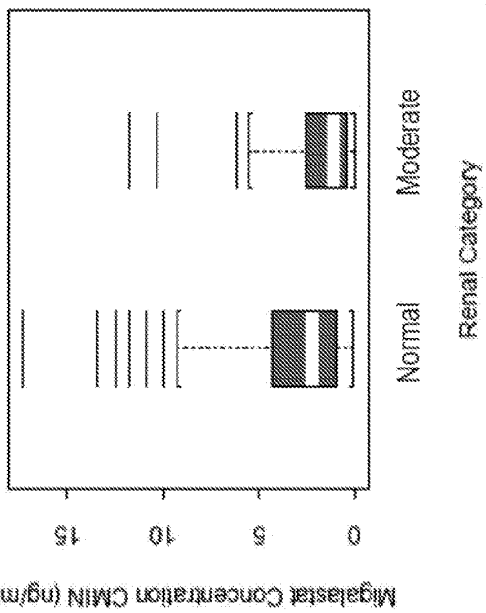
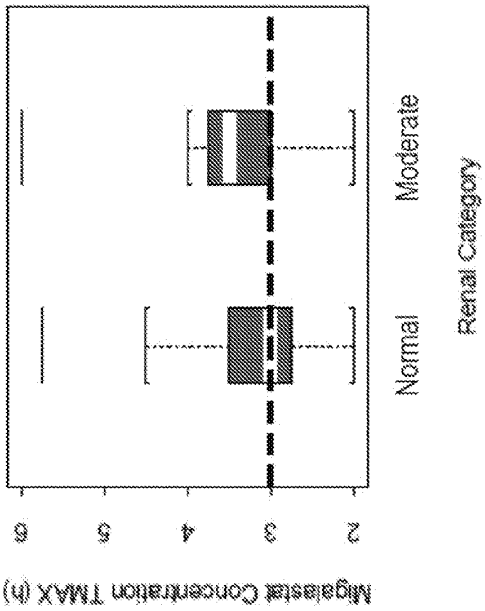


FIG. 12D



```
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FIG. 13A

```
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```

FIG. 13B

```
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tccgcccacctcggcctcccaaagtgctgggattacaggcatgagccaccgcgcctagcc     6060
tacaaatgttttgtaatagctcttgaggcccatcttggagttctcctttgctaaaacca     6120
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acagatttttttttgacggagtctcgctctgtcgcccaggctggagtgcagtggcaca     6360
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```

FIG. 13C

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FIG. 13D

```
gaatcgcttgaacccgggagtggaggttgcattgagctgagatcatgccacctcactcca    11580
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agaggcattttaaaga                                                12436
```

FIG. 13E

```
MQLRNPELHL GCALALRFLA LVSWDIPGAR ALDNGLARTP TMGWLHWERF MCNLDCQEEP   60
DSCISEKLFM EMAELMVSEG WKDAGYEYLC IDDCWMAPQR DSEGRLQADP QRFPHGIRQL  120
ANYVHSKGLK LGIYADVGNK TCAGFPGSFG YYDIDAQTFA DWGVDLLKFD GCYCDSLENL  180
ADGYKHMSLA LNRTGRSIVY SCEWPLYMWP FQKPNYTEIR QYCNHWRNFA DIDDSWKSIK  240
SILDWTSFNQ ERIVDVAGPG GWNDPDMLVI GNFGLSWNQQ VTQMALWAIM AAPLFMSNDL  300
RHISPQAKAL LQDKDVIAIN QDPLGKQGYQ LRQGDNFEVW ERPLSGLAWA VAMINRQEIG  360
GPRSYTIAVA SLGKGVACNP ACFITQLLPV KRKLGFYEWT SRLRSHINPT GTVLLQLENT  420
MQMSLKDLL                                                          429
```

FIG. 14

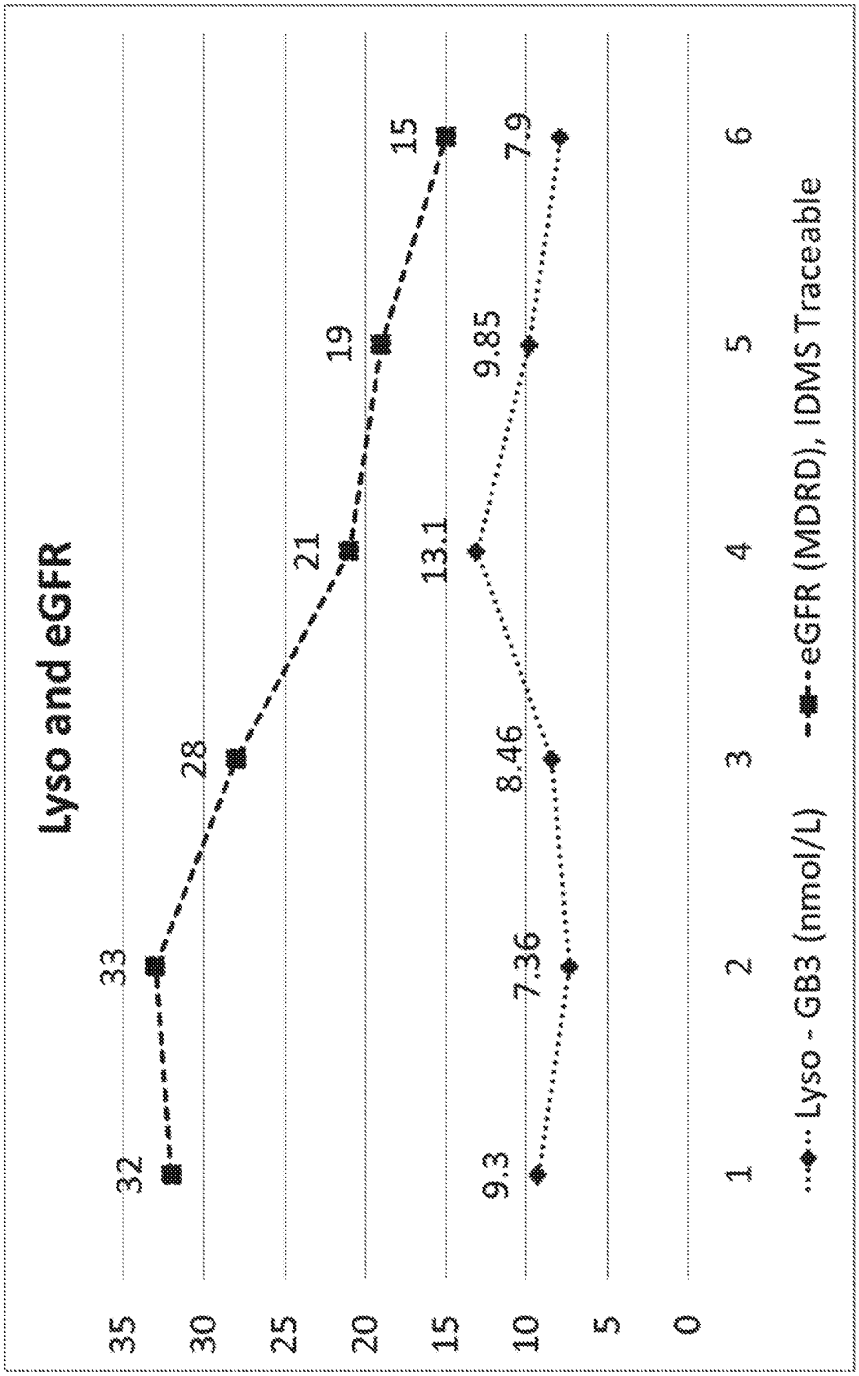

Lyso and eGFR
35
30
25
20
15
10
5
0
1
2
3
4
5
6
32
33
28
21
19
15
9.3
7.36
8.46
13.1
9.85
7.9
Lyso - GB3 (nmol/L)
eGFR (MDRD), IDMS Traceable

FIG. 15