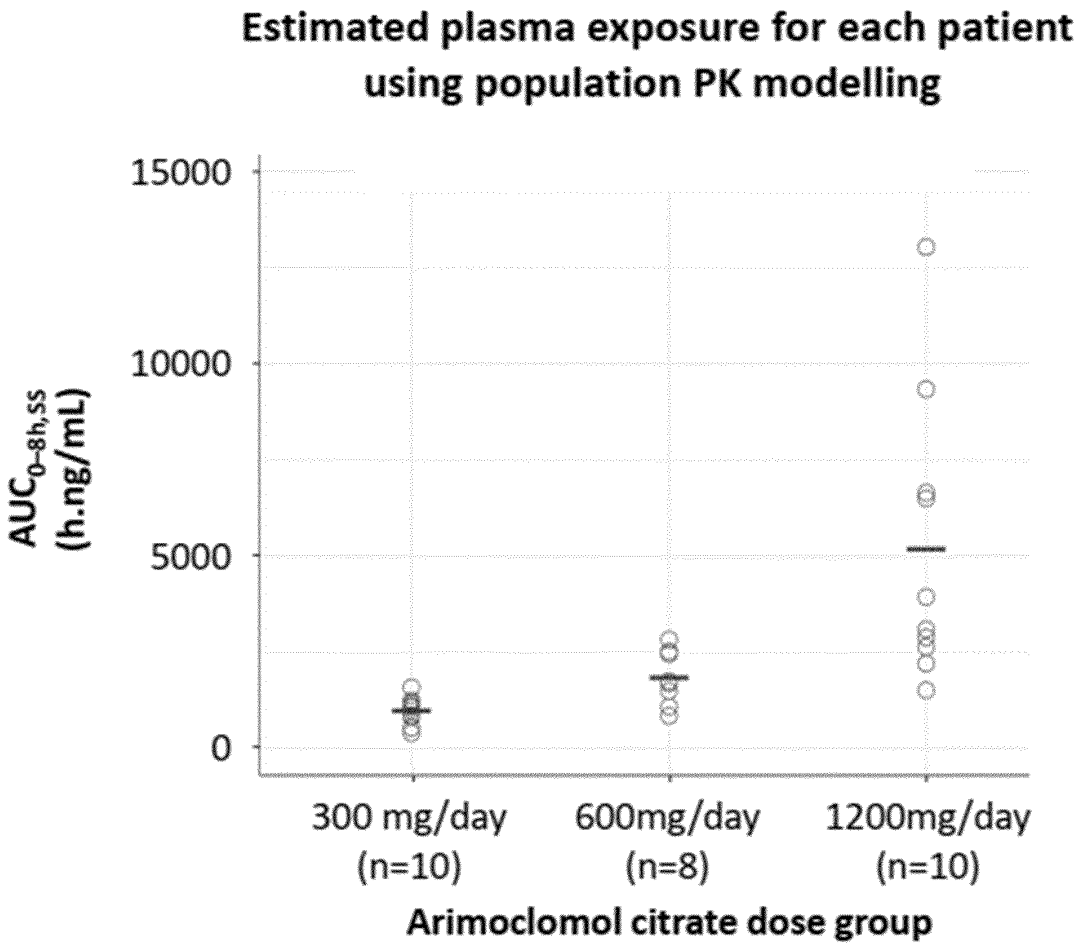

Estimated plasma exposure for each patient using population PK modelling
15000
10000
5000
0
AUC0–8h,SS (h.ng/mL)
300 mg/day (n=10)
600mg/day (n=8)
1200mg/day (n=10)
Arimoclomol citrate dose group

Fig. 1

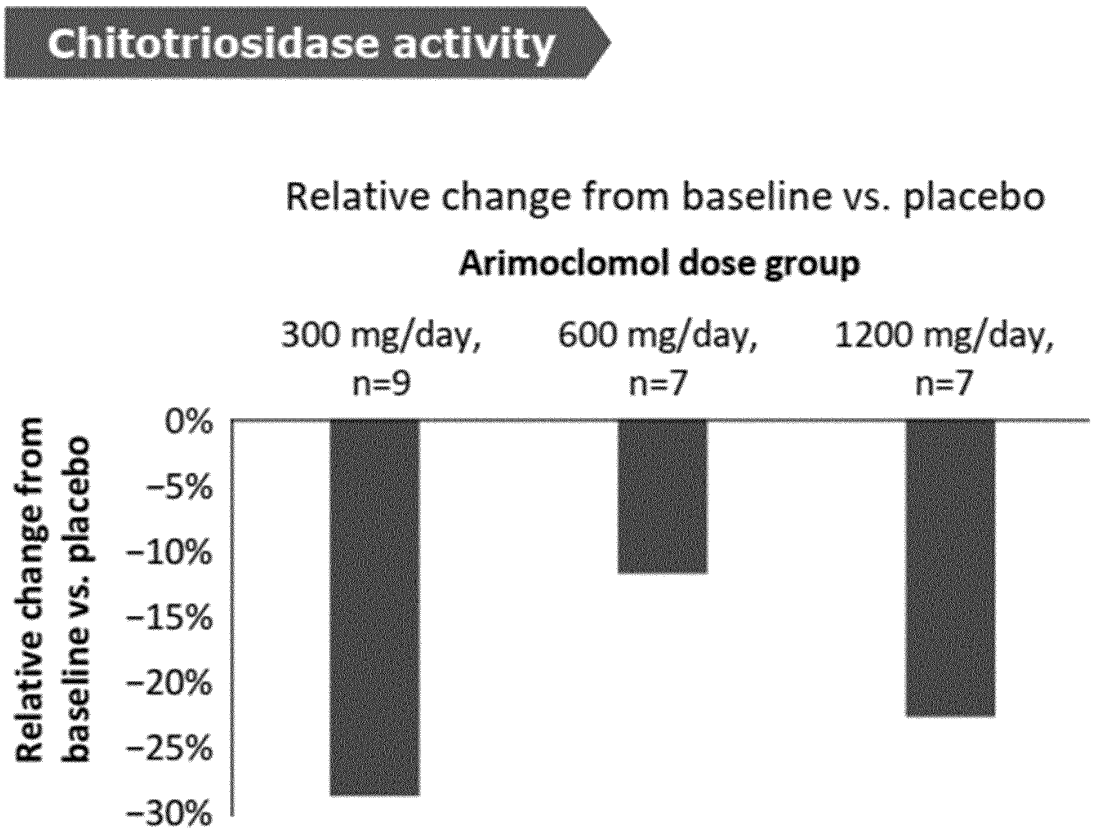

Chitotriosidase activity
Relative change from baseline vs. placebo
Arimoclomol dose group
300 mg/day, n=9
600 mg/day, n=7
1200 mg/day, n=7
Relative change from baseline vs. placebo
0%
−5%
−10%
−15%
−20%
−25%
−30%

Fig. 2

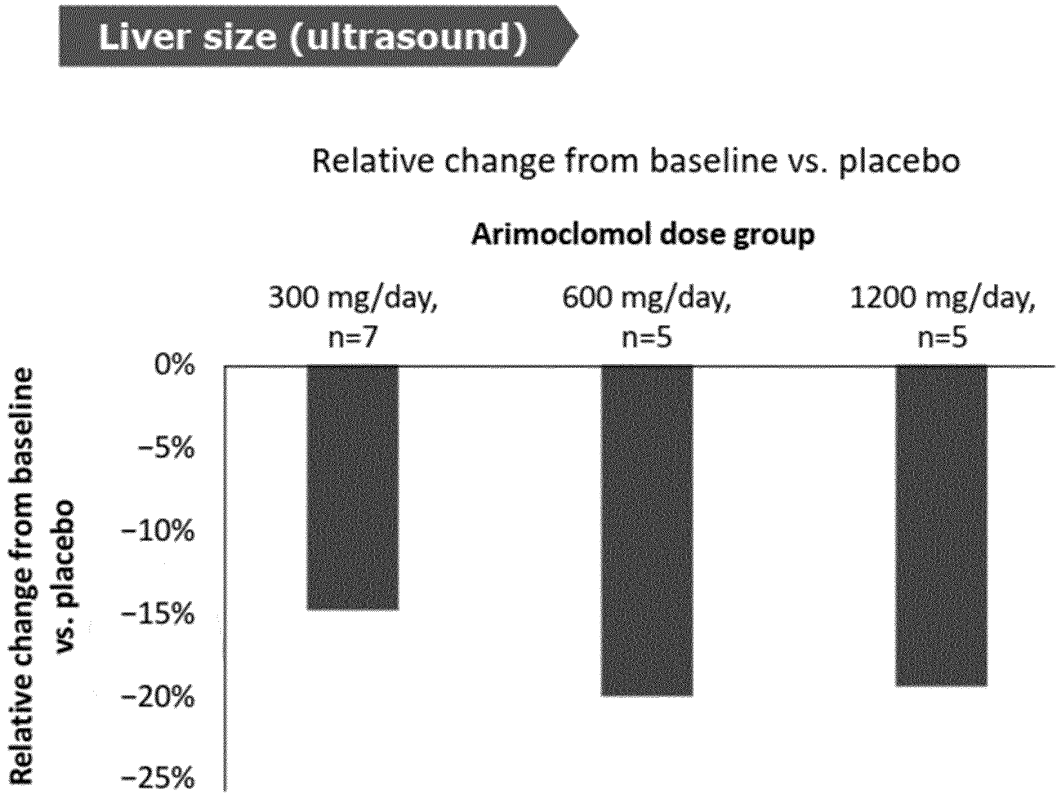

Liver size (ultrasound)
Relative change from baseline vs. placebo
Arimoclomol dose group
300 mg/day, n=7
600 mg/day, n=5
1200 mg/day, n=5
Relative change from baseline vs. placebo
0%
−5%
−10%
−15%
−20%
−25%

Fig. 3

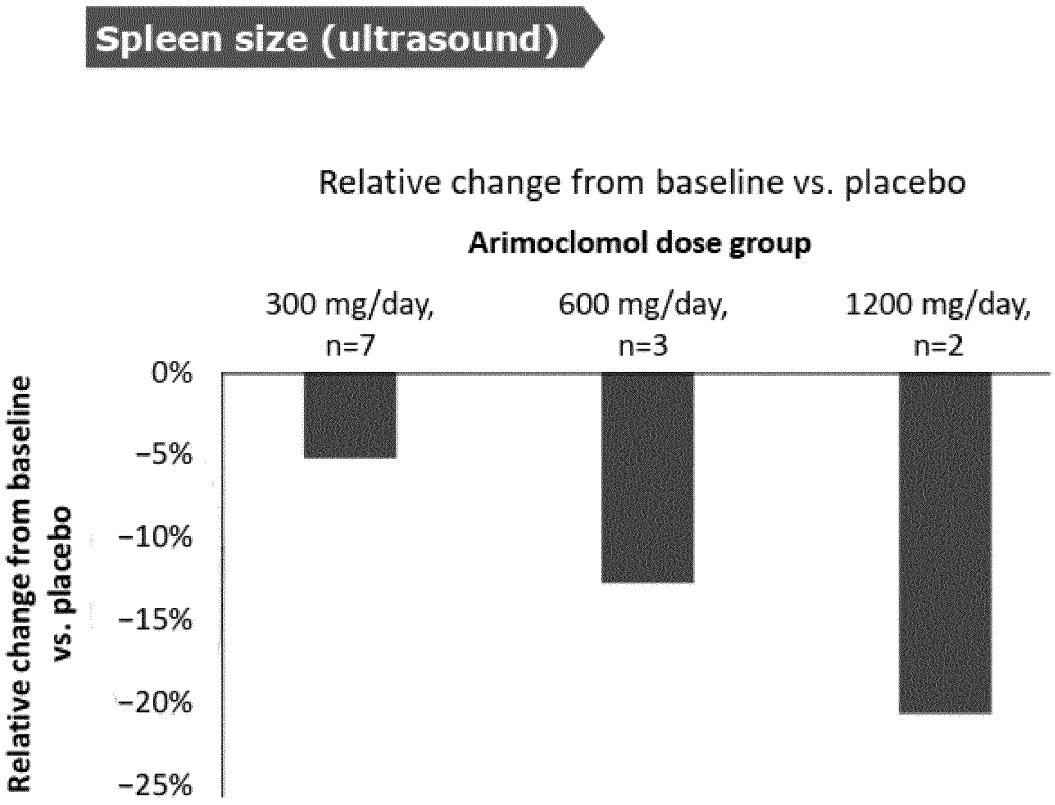

Spleen size (ultrasound)
Relative change from baseline vs. placebo
Arimoclomol dose group
300 mg/day, n=7
600 mg/day, n=3
1200 mg/day, n=2
Relative change from baseline vs. placebo
0%
−5%
−10%
−15%
−20%
−25%

Fig. 4

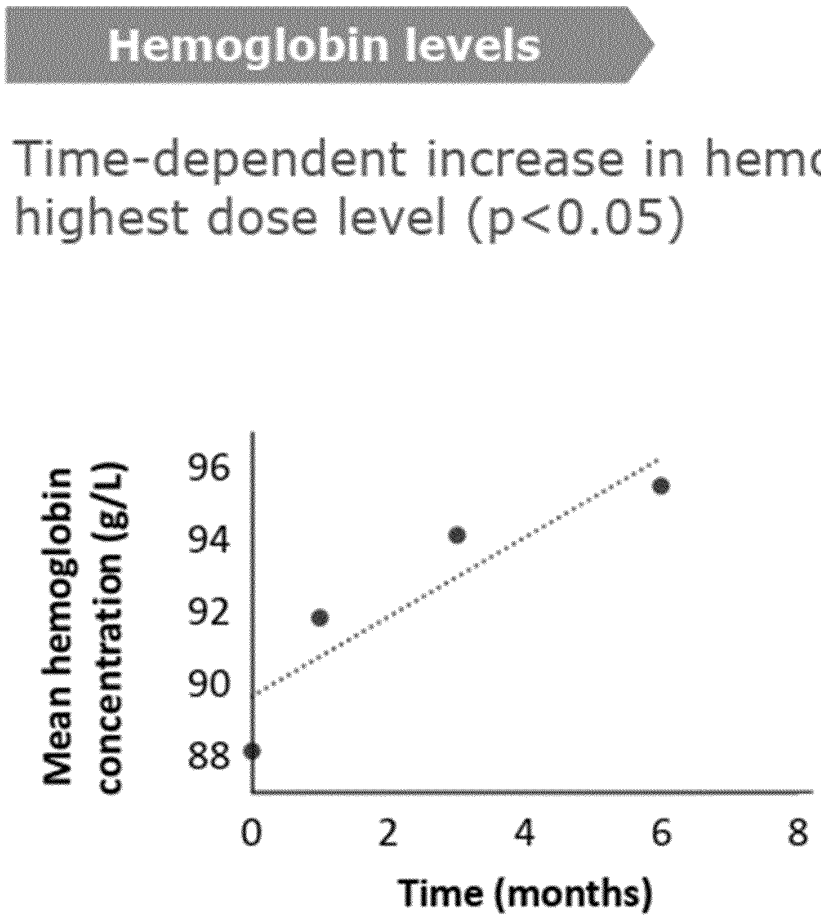

Hemoglobin levels
Time-dependent increase in hemoglobin at highest dose level (p<0.05)
Mean hemoglobin concentration (g/L)
96
94
92
90
88
0
2
4
6
8
Time (months)

Fig. 5

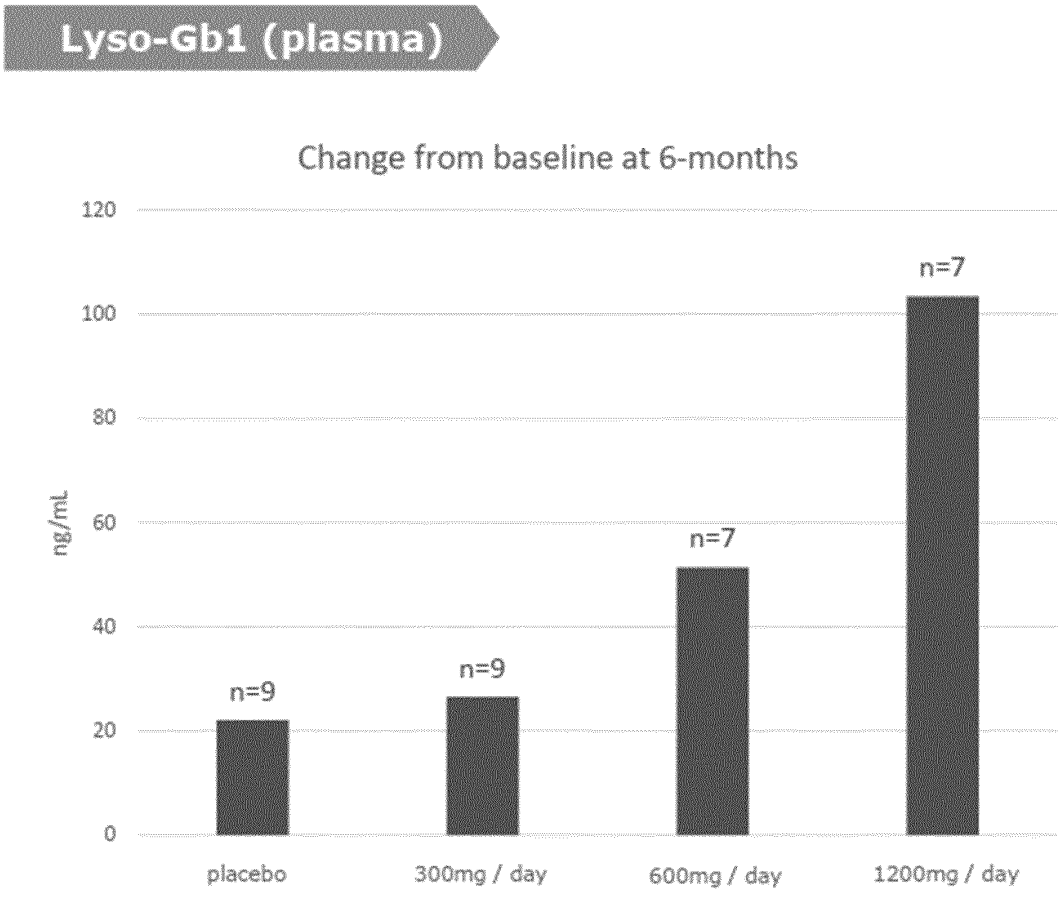

Lyso-Gb1 (plasma)
Change from baseline at 6-months
120
100
80
60
40
20
0
ng/mL
n=9
n=9
n=7
n=7
placebo
300mg / day
600mg / day
1200mg / day

Fig. 6