

**Supplemental Table 5.** Pre- and post-treatment levels of lyso-Gb<sub>3</sub> and analogues in plasma and urine and Gb<sub>3</sub> in urine in patients receiving treatment for the first time. Pairwise analyses were used. Lyso-Gb<sub>3</sub> and analogue values are expressed as median (Q1-Q3). Interval values are expressed as mean (SD) and represent the time between the date of measurement and the date of treatment. Lyso-Gb<sub>3</sub> (+50) data could not be analyzed due to many results below the limit of detection (LOD).

	agalsidase-beta			agalsidase-alfa			migalastat		
	before	after	p-value	before	after	p-value	before	after	p-value
Plasma (nM)									
n	26			5			1		
Lyso-Gb <sub>3</sub> (-28)	0.61 (0.13-1.14)	0.17 (<lod -0.44)	<0.001	0.57 (0.36-0.93)	0.08 (<lod -0.58)	0.06	0.44 (-)	0.16 (-)	
Lyso-Gb <sub>3</sub> (-2)	4.25 (2.18-6.27)	3.03 (1.66-4.15)	<0.001	3.88 (3.02-7.64)	3.06 (1.41-5.43)	0.06	4.82 (-)	3.08 (-)	
Lyso-Gb <sub>3</sub>	12.18 (8.86-20.42)	9.62 (6.11-14.71)	<0.001	15.12 (10.12-21.62)	8.43 (6.47-17.45)	0.06	15.20 (-)	10.48 (-)	
Lyso-Gb <sub>3</sub> (+16)	1.06 (0.44-2.63)	0.54 (<lod -1.14)	<0.001	0.83 (0.62-2.10)	0.28 (0.11-1.26)	0.06	0.92 (-)	0.65 (-)	
Lyso-Gb <sub>3</sub> (+18)	0.31 (<lod -0.62)	<lod (<lod -0.21)	<0.001	1.08 (0.56-1.46)	0.67 (0.30-0.84)	0.13	0.76 (-)	0.54 (-)	
Lyso-Gb <sub>3</sub> (+34)	0.14 (<lod -0.69)	<lod (<lod -0.06)	0.002	0.69 (0.41-0.85)	0.24 (<lod -0.31)	0.06	0.36 (-)	<lod (-)	
Lyso-Gb <sub>3</sub> (+50)	<lod (<lod -<lod)	<lod (<lod -<lod)	-	<lod (<lod -<lod)	<lod (<lod -<lod)	-	<lod (-)	<lod (-)	
Urine (pM/mM cre)									
n	10			2 <sup>¶</sup>			1		
LysoGb <sub>3</sub> (-28)	16.26 (7.72-33.30)	6.25 (9.39 -30.52)	0.005	22.42 (15.66-28.31)	3.91 (<lod -16.21)	nd	23.64 (-)	26.25 (-)	
Lyso-Gb <sub>3</sub> (-12)	89.78 (44.17-165.69)	39.80 (21.38-82.29)	0.005	164.06 (82.77-173.66)	91.84 (36.17-111.92)	nd	77.11 (-)	82.60 (-)	
Lyso-Gb <sub>3</sub> (-2)	22.29 (12.81-44.98)	13.72 (5.58-17.39)	0.005	22.98 (12.58-32.24)	14.39 (6.71-25.22)	nd	30.34 (-)	34.25 (-)	
Lyso-Gb <sub>3</sub>	26.88 (9.35-38.68)	25.00 (9.39-30.52)	0.14	23.31 (3.57-41.74)	11.91 (4.22-23.98)	nd	35.89 (-)	46.09 (-)	
Lyso-Gb <sub>3</sub> (+14)	24.05 (14.40-64.25)	13.02 (6.02-32.46)	0.005	42.36 (27.85-46.03)	20.08 (12.01-28.46)	nd	27.30 (-)	34.85 (-)	
Lyso-Gb <sub>3</sub> (+16)	188.89 (95.68-381.87)	116.34 (74.96-216.00)	0.005	379.88 (168.03-412.13)	210.68 (72.95-253.42)	nd	251.77 (-)	339.92 (-)	
Lyso-Gb <sub>3</sub> (+34)	130.23 (72.73-190.62)	69.27 (44.13-93.13)	0.005	153.23 (109.75-130.43)	63.97 (42.26-64.03)	nd	135.35 (-)	191.39 (-)	
Lyso-Gb <sub>3</sub> (+50)	60.51 (31.05-101.00)	41.63 (32.87-74.76)	0.09	95.30 (47.60-105.69)	62.08 (28.13-75.33)	nd	70.70 (-)	101.99 (-)	
Gb <sub>3</sub> (µg/mmol creatinine)	32.25 (15.27-47.72)	14.05 (10.47-20.72)	0.03	28.75 (24.20-33.30)	14.95 (9.60-20.30)	nd			

nd: not done due to the small sample size

<sup>¶</sup> Levels of lyso-Gb<sub>3</sub> and analogues in urine of the 2 patients in pM/mM cre: Pre-treatment: Lyso-Gb<sub>3</sub>(-28) 23.95/20.88, Lyso-Gb<sub>3</sub>(-12) 217.76/110.36, Lyso-Gb<sub>3</sub>(-2) 29.19/16.77, Lyso-Gb<sub>3</sub> 41.86/4.76, Lyso-Gb<sub>3</sub>(+14) 47.58/37.13, Lyso-Gb<sub>3</sub>(+16) 535.71/224.04, Lyso-Gb<sub>3</sub>(+34) 160.12/146.33, Lyso-Gb<sub>3</sub>(+50) 127.13/63.46. Post-treatment: Lyso-Gb<sub>3</sub>(-28) <lod/7.82, Lyso-Gb<sub>3</sub>(-12) 135.44/48.23, Lyso-Gb<sub>3</sub>(-2) 19.83/8.95, Lyso-Gb<sub>3</sub> 18.18/5.63, Lyso-Gb<sub>3</sub>(+14) 24.15/16.01, Lyso-Gb<sub>3</sub>(+16) 324.1/97.26, Lyso-Gb<sub>3</sub>(+34) 71.58/56.35, Lyso-Gb<sub>3</sub>(+50) 86.65/37.5