

Table S1. Primary goal attainment (GAS T score) at Weeks 6 and 16 by dose (mITT population)

	8 U/kg	16 U/kg
Cycle 1		
Week 6	n=66, 52.6 ± 10.1	n=70, 52.0 ± 9.6
Week 16	n=67, 54.2 ± 9.7	n=69, 55.1 ± 10.1
Cycle 2		
Week 6	n=84, 51.9 ± 9.5	n=87, 53.4 ± 9.1
Week 16	n=81, 52.0 ± 9.5	n=84, 55.0 ± 9.6
Cycle 3		
Week 6	n=45, 49.1 ± 10.4	n=55, 48.7 ± 8.7
Week 16	n=39, 50.5 ± 8.8	n=53, 49.7 ± 9.7
Cycle 4		
Week 6	n=20, 43.7 ± 8.4	n=31, 48.1 ± 9.0
Week 16	n=19, 48.9 ± 8.1	n=31, 50.0 ± 9.4

Data are mean ±SD

Fig. S1. Goal attainment for children who required four treatment cycles during the study and had a GAS assessment at Week 6 and 16

