

Secondary efficacy outcome 5: Height

Table 6.6-18: Height – Completeness of data (ITT)

Time-point	Height	CSII	MDI	Total
Height (0m)	N	144	149	293
	Measured	124 (86.1%)	132 (88.6%)	256 (87.4%)
	Missing data	20 (13.9%)	17 (11.4%)	37 (12.6%)
Height (12m)	N	144	149	293
	Measured	142 (98.6%)	138 (92.6%)	280 (95.6%)
	Missing data	1 (0.7%)	6 (4%)	7 (2.4%)
	WD from study prior to 12 months	1 (0.7%)	5 (3.4%)	6 (2%)
Height (at both 0m and 12m)	N	144	149	293
	Measured	122 (84.7%)	122 (81.9%)	244 (83.3%)
	Missing data	21 (14.6%)	22 (14.8%)	43 (14.7%)
	WD from study prior to 12 months	1 (0.7%)	5 (3.4%)	6 (2%)

Table 6.6-19: Height – Results (ITT)

Time-point	Height	CSII	MDI	Total	Mean Difference (95% CI)	P-value
Height (0m)	N	124	132	256	-0.1 (-0.2, 0)*	0.10*
	Mean (SD)	0.3 (1.1)	0.3 (1)	0.3 (1)		
	Median (IQR)	0.1 (-0.4, 1.1)	0.4 (-0.3, 1.1)	0.3 (-0.3, 1.1)		
	(Min, Max)	(-2.3, 3.3)	(-2.9, 2.4)	(-2.9, 3.3)		
Height (12m)	N	142	138	280		
	Mean (SD)	0.2 (1.1)	0.3 (1)	0.3 (1)		
	Median (IQR)	0.1 (-0.6, 1)	0.3 (-0.3, 1)	0.2 (-0.5, 1)		
	(Min, Max)	(-2.1, 3.5)	(-2.7, 2.3)	(-2.7, 3.5)		
Height (12m - 0m)	N	122	122	244		
	Mean (SD)	-0.1 (0.5)	0 (0.4)	0 (0.4)		
	Median (IQR)	0 (-0.2, 0.2)	0 (-0.2, 0.2)	0 (-0.2, 0.2)		
	(Min, Max)	(-2.8, 0.7)	(-1.7, 1.6)	(-2.8, 1.6)		

* Primary analysis used (12m HEIGHT SDS – baseline HEIGHT SDS) as the outcome variable with baseline HEIGHT SDS, age strata, treatment and centre (random effect) as covariates. A secondary analysis uses 12m HEIGHT SDS as the outcome with the same covariates. Both analyses give the same treatment difference, 95% confidence interval and p-value.