

**Table S3.** LS mean change from baseline to year 2 (MorCAP) or week 120 (MOR-005) based on a repeated-measures ANCOVA model<sup>a</sup>.

<b>Measure</b>	<b>Study</b>	<b>Analysis</b>	<b>N</b>	<b>LS mean change</b>	<b>SE</b>	<b>P value<sup>b</sup></b>
<b>6MWT, m</b>	MorCAP	ITT	9	6.5	15.9	.68
	MOR-005	ITT	33	30.5	10.8	.0064
		MPP	30	34.9	11.7	.0042
<b>3MSCT, stairs/min</b>	MorCAP	ITT	9	4.0	4.7	.40
	MOR-005	ITT	33	5.9	1.7	.001
		MPP	30	6.7	1.8	.0005
<b>uKS % change</b>	MorCAP	ITT	4	46.1	58.1	.43
	MOR-005	ITT	30	-65.6	2.4	<.0001
		MPP	27	-66.3	2.7	<.0001
<b>FVC, L</b>	MorCAP	ITT	9	-0.017	0.04	.66
	MOR-005	ITT	31	-0.031	0.03	.32
		MPP	28	-0.007	0.03	.83
<b>FEV<sub>1</sub>, L</b>	MorCAP	ITT	9	-0.042	0.05	.42
	MOR-005	ITT	32	-0.034	0.02	.15
		MPP	29	-0.009	0.02	.69
<b>MVV change, L/min</b>	MorCAP	ITT	7	-0.86	2.9	.77
	MOR-005	ITT	30	0.90	2.0	.65
		MPP	27	1.65	2.2	.45
<b>Caregiver assistance change</b>	MorCAP	ITT	10	0.29	1.3	.82
	MOR-005	ITT	33	-1.02	0.9	.26
		MPP	30	-1.24	1.0	.21
<b>Mobility change</b>	MorCAP	ITT	10	-0.20	0.5	.69
	MOR-005	ITT	33	-0.76	0.2	.002
		MPP	30	-0.81	0.3	.003
<b>Self-care</b>	MorCAP	ITT	10	0.53	0.3	.071
	MOR-005	ITT	33	-0.43	0.2	.010
		MPP	30	-0.58	0.2	.001

3MSCT, 3-minute stair climb test; 6MWT, 6-minute walk test; ANCOVA, analysis of covariance; FEV<sub>1</sub>, forced expiratory volume in 1 second; FVC, forced vital capacity; ITT, intent to treat; LS, least squares; MPP, modified per-protocol; MVV, maximal voluntary ventilation; uKS, urinary keratan sulfate.

<sup>a</sup>For 6MWT, the model included baseline 6MWT category (< or ≥200 m), treatment, time point, and treatment and time point interaction. For all other assessments, baseline measurement replaced baseline 6MWT category, except for 3MSCT and uKS, which included both terms.

<sup>b</sup>P values were calculated for LS mean assessments at year 2 or week 120 vs baseline.