

**Table S1.** Descriptive statistics on change from baseline to year 2 (MorCAP) or week 120 (MOR-005).

<b>Measure</b>	<b>Study</b>	<b>Analysis</b>	<b>N</b>	<b>Mean change</b>	<b>SE</b>
<b>6MWT, m</b>	MorCAP	ITT	9	4.3	10.5
	MOR-005	ITT	33	29.6	13.8
		MPP	30	34.1	14.7
<b>3MSCT, stairs/min</b>	MorCAP	ITT	9	3.4	5.2
	MOR-005	ITT	33	5.8	2.0
		MPP	30	6.6	2.1
<b>uKS , <math>\mu\text{g}/\text{mg}^{\text{a}}</math></b>	MorCAP	ITT	4	3.0	1.9
	MOR-005	ITT	30	-6.4	0.9
		MPP	27	-6.6	0.9
<b>uKS , % change<sup>a</sup></b>	MorCAP	ITT	4	72.3	37.3
	MOR-005	ITT	30	-65.4	2.2
		MPP	27	-66.0	2.4
<b>FVC, L</b>	MorCAP	ITT	9	-0.002	0.05
	MOR-005	ITT	31	0.0	0.04
		MPP	28	0.0	0.04
<b>FVC % change</b>	MorCAP	ITT	9	-2.0	3.3
	MOR-005	ITT	31	-2.6	2.7
		MPP	28	-0.7	2.8
<b>FEV<sub>1</sub>, L</b>	MorCAP	ITT	9	-0.039	0.047
	MOR-005	ITT	32	0.0	0.04
		MPP	29	0.0	0.02
<b>FEV<sub>1</sub> % change</b>	MorCAP	ITT	9	-4.2	3.8
	MOR-005	ITT	32	-1.9	2.9
		MPP	29	0.5	2.8
<b>MVV, L/min</b>	MorCAP	ITT	7	-1.40	3.8
	MOR-005	ITT	30	0.9	2.2
		MPP	27	1.7	2.3
<b>MVV % change</b>	MorCAP	ITT	7	-1.9	7.7
	MOR-005	ITT	30	1.7	4.5
		MPP	27	3.7	4.6
<b>Self-care</b>	MorCAP	ITT	10	0.5	0.5
	MOR-005	ITT	33	-0.4	0.2
		MPP	30	-0.6	0.2
<b>Caregiver assistance change</b>	MorCAP	ITT	10	0.4	2.3
	MOR-005	ITT	33	-1.2	1.1
		MPP	30	-1.3	1.2
<b>Mobility change</b>	MorCAP	ITT	10	-0.3	1.0
	MOR-005	ITT	33	-0.8	0.3
		MPP	30	-0.8	0.3

3MSCT, 3-minute stair climb test; 6MWT, 6-minute walk test; FEV<sub>1</sub>, forced expiratory volume in 1 second; FVC, forced vital capacity; HAQ, Health Assessment Questionnaire; ITT, intent to treat; MPP, modified per-protocol; MVV, maximal voluntary ventilation; uKS, urinary keratan sulfate.

<sup>a</sup>Normalized uKS is calculated as uKS divided by urine creatinine.