

SUPPLEMENTARY FILE

Table S1. Stratified pulmonary exacerbations data for the 2014 UK ivacaftor and comparator safety cohorts

Strata	Ivacaftor safety cohort		Comparator safety cohort		RR	95% CI for RR/ Fisher's exact p value ^a
	n	Risk, %	n	Risk, %		
Age, y						
0 to <6	1	12.5	18	45.0	0.28	p=0.12
≥6 to <12	16	22.2	129	39.7	0.56	0.36–0.88
≥12 to <18	22	29.3	250	58.7	0.50	0.35–0.72
≥18	101	39.5	760	59.5	0.66	0.56–0.77
ppFEV ₁						
<40	27	71.1	234	88.0	0.81	0.66–1.00
≥40 to <70	45	50.6	447	69.2	0.73	0.59–0.90
≥70	64	23.4	412	39.3	0.59	0.47–0.74

^aFisher's exact p values are shown when the expected value is <5 in at least 1 cell of the contingency table.

CI, confidence interval; ppFEV₁, percent predicted forced expiratory volume in 1 second; RR, relative risk.

Table S2. Stratified hospitalisations for pulmonary exacerbations data for the 2014 UK ivacaftor and comparator safety cohorts

Strata	Ivacaftor safety cohort		Comparator safety cohort		RR	95% CI for RR/ Fisher's exact p value ^a
	n	Risk, %	n	Risk, %		
Age, y						
0 to <6	1	12.5	16	40.0	0.31	p=0.23
≥6 to <12	15	20.8	115	35.4	0.59	0.37–0.95
≥12 to <18	19	25.3	227	53.3	0.48	0.32–0.72
≥18	72	28.1	579	45.3	0.62	0.51–0.76
ppFEV ₁						
<40	16	42.1	194	72.9	0.58	0.40–0.85
≥40 to <70	37	41.6	352	54.5	0.76	0.59–0.98
≥70	50	18.3	328	31.3	0.58	0.44–0.76

^aFisher's exact p values are shown when the expected value is <5 in at least 1 cell of the contingency table.

CI, confidence interval, ppFEV₁, percent predicted forced expiratory volume in 1 second; RR, relative risk.

Table S3. Stratified hospitalisations data for the 2014 US ivacaftor and comparator safety cohorts

Strata	Ivacaftor safety cohort		Comparator safety cohort		RR	95% CI for RR/ Fisher's exact p value ^a
	n	Risk, %	n	Risk, %		
Age, y						
0 to <6	2	4.8	55	26.8	0.18	0.05–0.70
≥6 to <12	25	11.6	338	30.5	0.38	0.26–0.56
≥12 to <18	85	31.7	634	49.7	0.64	0.53–0.77
≥18	234	32.0	1644	45.6	0.70	0.63–0.79
ppFEV ₁						
<40	56	52.8	532	73.9	0.71	0.59–0.86
≥40 to <70	121	39.9	917	55.9	0.71	0.62–0.83
≥70	165	20.5	1135	31.5	0.65	0.56–0.75

^aFisher's exact p values are shown when the expected value is <5 in at least 1 cell of the contingency table.

CI, confidence interval; ppFEV₁, percent predicted forced expiratory volume in 1 second; RR, relative risk.

Table S4. Stratified pulmonary exacerbations data for the 2014 US ivacaftor and comparator safety cohorts

Strata	Ivacaftor safety cohort		Comparator safety cohort		RR	95% CI for RR/ Fisher's exact p value ^a
	n	Risk, %	n	Risk, %		
Age, y						
0 to <6	2	4.8	49	23.9	0.20	0.05–0.79
≥6 to <12	20	9.3	307	27.7	0.34	0.22–0.52
≥12 to <18	73	27.2	606	47.5	0.57	0.47–0.70
≥18	254	34.8	1722	47.7	0.73	0.66–0.81
ppFEV ₁						
<40	66	62.3	574	79.7	0.78	0.67–0.91
≥40 to <70	135	44.6	965	58.8	0.76	0.66–0.86
≥70	143	17.7	1068	29.7	0.60	0.51–0.70

^aFisher's exact p values are shown when the expected value is <5 in at least 1 cell of the contingency table.

CI, confidence interval; ppFEV₁, percent predicted forced expiratory volume in 1 second; RR, relative risk.