Mario 1 – Study IPI-549-01 Manuscript Supplementary Data and Methods

Table S2. Summary of anti-tumor activity endpoints in patients treated with eganelisib monotherapy and combination therapy

Category	Monotherapy (N=39) n (%)	Combination therapy (N=180) n (%)
n	37	169
ORR	1 (2.7)	12 (7.1)
95% exact binomial CI	[0.1, 14.2]	[3.7, 12.1]
DCR (CR+PR+SD) ^a		L / J
n	37	169
DCR	15 (40.5)	61 (36.1)
95% exact binomial CI	[24.8, 57.9]	[28.9, 43.8]
Best overall response ^a		
n	39	180
CR	0	1 (0.6)
PR	1 (2.6)	11 (6.1)
SD	14 (35.9)	49 (27.2)
PD	16 (41.0)	79 (43.9)
NE	6 (15.4)	29 (16.1)
CBR-24 ^{b,c}		
n	37	169
CB	5 (13.5)	25 (14.8)
95% exact binomial CI	[4.5, 28.8]	[9.8, 21.1]
CBR-16 ^{b,c}		
n	37	169
CB	7 (18.9)	31 (18.3)
95% exact binomial CI	[8.0, 35.2]	[12.8, 25.0]
TTR, months ^d		
Median (95% CI)	11.1 [NA, NA]	1.8 [1.8, 2.3]
DOR, months ^d		
Patients with PD or died, n	1	10
Censored, n	0	4
Median (95% CI)	9.5 [NA, NA]	9.2 [3.8, 11.4]
DOT, months		
Patients with treatment failure ^e , n	30	157
Censored, n	9	23
Median (95% CI)	2.0 [1.9, 4.7]	2.1 [1.9, 2.7]
PFS, months		
Patients who died or had PD, n	30	148
Censored, n	9	32
Median (95% CI)	1.9 [1.7, 3.7]	1.9 [1.9, 3.2]
OS, months		
Patients who died, n	19	75
Censored, n	20	105
Median (95% CI)	14.9 [7.0, 19.3]	11.7 [8.6, 14.1]

Abbreviations: AE, adverse event; CB, clinical benefit; CBR, clinical benefit rate; CI, confidence interval; CR, complete response; DCR, disease control rate; DOR, duration of response; DOT, duration of treatment; NA, not applicable; NE, not evaluable; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; PD, progressive disease; PR, partial response; SD, stable disease; TTR; time to response. Time to event parameters were estimated using Kaplan-Meier method.

^aBased on investigator assessment.

^bCalculated based on response evaluable set within the monotherapy or combination therapy cohort.

CBR is defined as achieving confirmed CR, PR or SD of at least 24 weeks (CBR-24) or 16 weeks (CBR-16) from cycle 1 day 1.

^dAnalyzed for responders only.

^eDefined as discontinuation due to AEs, progressive disease, and death.