Supplementary Content: Safety and Efficacy of Buparlisib (BKM120) in Patients With PI3K Pathway-Activated Non-Small Cell Lung Cancer (NSCLC): Results From the Phase II BASALT-1 Study

SUPPLEMENTARY TABLE 1. Summary of PI3K Pathway Alterations at Pre-Screening Stage

PI3K Pathway Status	Squamous (*n* = 668)	Non-Squamous (*n* = 574)	Total (*N* = 1242)[a]
PI3K pathway activation[b]	103 (15.4)	65 (11.3)	168 (13.5)
***PIK3CA* mutation status, *n* (%)**			
Patients with confirmed status[c]	388	349	737
PIK3CA mutation	26 (6.7)	22 (6.3)	48 (6.5)
PIK3CA wild-type	362 (93.3)	327 (93.7)	689 (93.5)
Unknown	69	95	164
Missing	211	130	341
PIK3CA mutation only[d]	21 (5.4)	15 (4.3)	36 (4.9)
***PTEN* mutation status, *n* (%)**			
Patients with confirmed status[c]	394	342	736
PTEN mutation	25 (6.3)	36 (10.5)	61 (8.3)
PTEN wild-type	369 (93.7)	306 (89.5)	675 (91.7)
Unknown	61	90	151
Missing	213	142	355
PTEN mutation only[d]	18 (4.6)	29 (8.5)	47 (6.4)
PTEN expression, *n* (%)			
Patients with confirmed status[c]	444	358	802
PTEN negative (<10% IHC)	64 (14.4)	16 (4.5)	80 (10.0)
PTEN positive	380 (85.6)	342 (95.5)	722 (90.0)
Unknown	17	32	49
Missing	207	184	395
PTEN negative only[d]	53 (11.9)	12 (3.4)	65 (8.1)

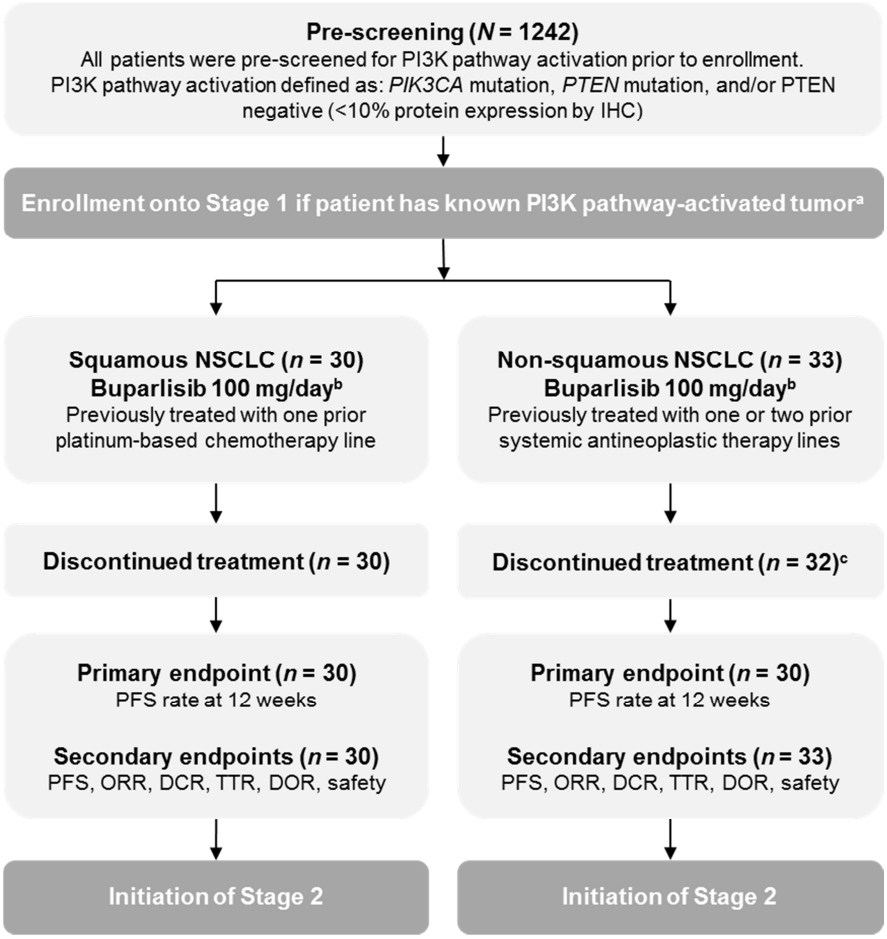
[a]Four patients were recorded with 'unknown' histology.
[b]PI3K activation defined as *PIK3CA* mutation, *PTEN* mutation, or PTEN negative (<10% protein expression by IHC).
[c]Patients with confirmed alteration status were used to calculate percentages.
[d]Includes only patients with the specified alteration, wild-type/positive for the other two markers.
Unknown, sample analyzed with inconclusive result; missing, sample was not analyzed for that marker.
IHC, immunohistochemistry; PI3K, phosphatidylinositol 3-kinase; PTEN, phosphatase and tensin homolog.

SUPPLEMENTARY TABLE 2. Summary of Adverse Events Suspected to Be Related to Study Drug

	Squamous *n* = 30		Non-Squamous *n* = 33	
Adverse event, *n* (%)	Grade 3/4	All grades	Grade 3/4	All grades
Hyperglycemia	7 (23.3)	11 (36.7)	4 (12.1)	10 (30.3)
Pruritus	1 (3.3)	10 (33.3)	0	4 (12.1)
Diarrhea	0	9 (30.0)	1 (3.0)	8 (24.2)
Nausea	0	9 (30.0)	1 (3.0)	9 (27.3)
Rash	1 (3.3)	8 (26.7)	2 (6.1)	7 (21.2)
Fatigue	2 (6.7)	7 (23.3)	1 (3.0)	4 (12.1)
Decreased appetite	0	7 (23.3)	0	9 (27.3)
Asthenia	2 (6.7)	5 (16.7)	2 (6.1)	10 (30.3)
Anemia	1 (3.3)	4 (13.3)	1 (3.0)	2 (6.1)
Abdominal pain	0	3 (10.0)	1 (3.0)	1 (3.0)
Constipation	0	3 (10.0)	0	2 (6.1)
Vomiting	0	3 (10.0)	0	3 (9.1)
Increased ALT	1 (3.3)	3 (10.0)	5 (15.2)	6 (18.2)
Increased AST	1 (3.3)	3 (10.0)	4 (12.1)	6 (18.2)
Anxiety	1 (3.3)	2 (6.7)	0	7 (21.2)
Depression	0	2 (6.7)	1 (3.0)	6 (18.2)
Dry skin	0	2 (6.7)	0	5 (15.2)
Dysgeusia	0	2 (6.7)	0	4 (12.1)
Stomatitis	0	2 (6.7)	0	4 (12.1)

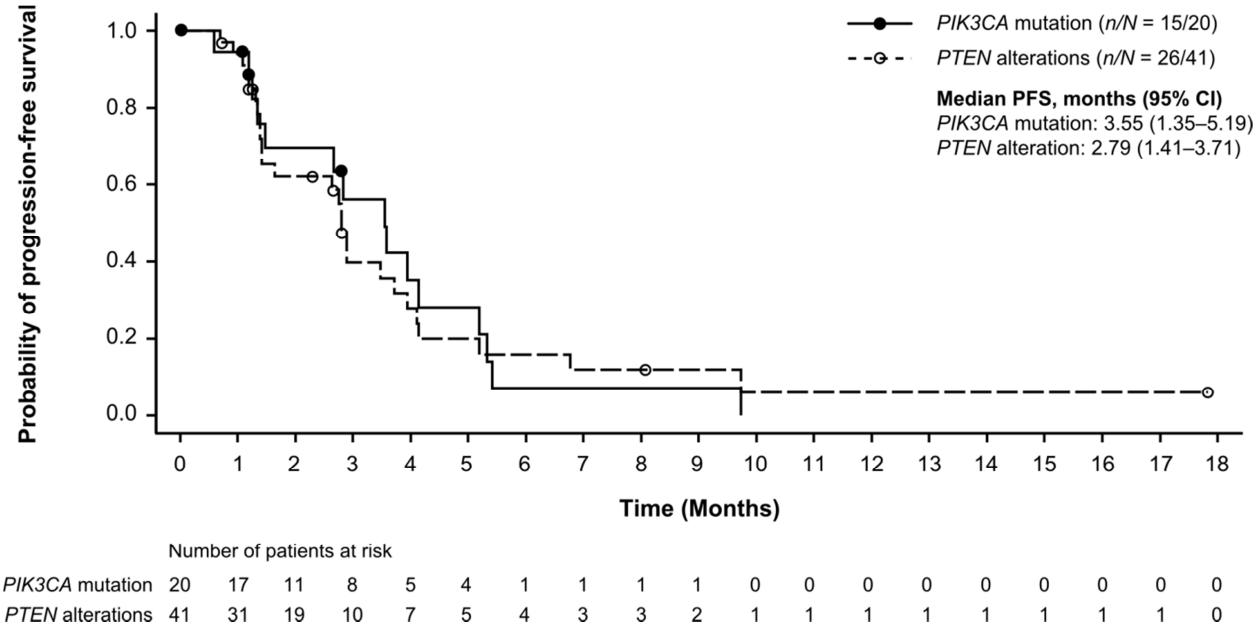
Adverse events were described according to MedDRA v17.0, and are listed in order of descending frequency in the squamous group 'All grades' column.
ALT, alanine aminotransferase; AST, aspartate aminotransferase; MedDRA, Medical Dictionary for Regulatory Activities.

SUPPLEMENTARY FIGURE 1. Study Schema



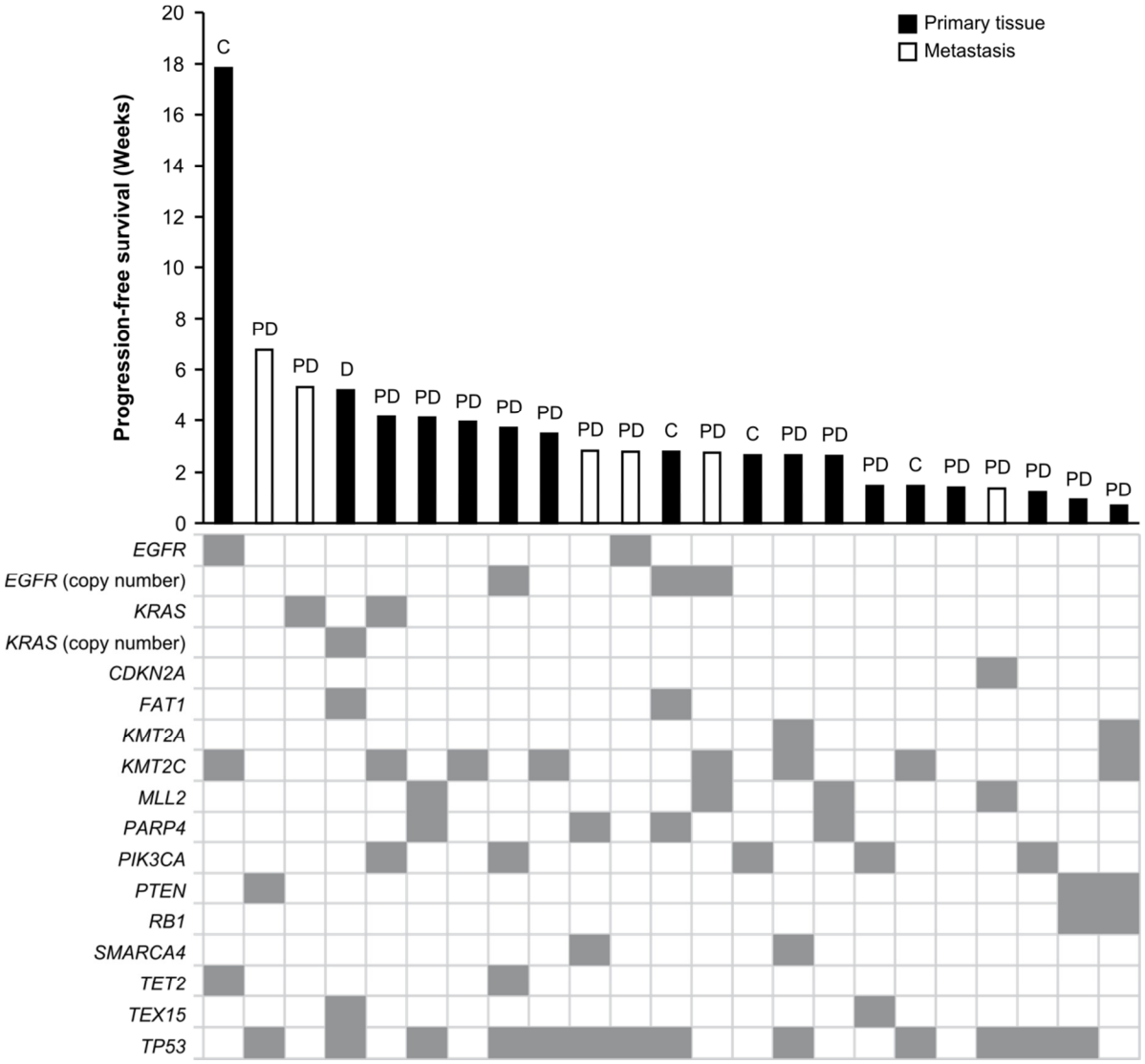
[a]BASALT-1 is a two-stage trial; data reported here are for Stage 1 only.
[b]Continuous 21-day cycles.
[c]One patient continued to receive treatment at the cut-off date (June 5, 2014).
DCR, disease control rate; DOR, duration of response; IHC, immunohistochemistry; NSCLC, non-small cell lung cancer; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; PI3K, phosphatidylinositol 3-kinase; PTEN, phosphatase and tensin homolog; TTR, time to response.

SUPPLEMENTARY FIGURE 2. Kaplan–Meier Plot of Progression-Free Survival in *PIK3CA*-Mutant and *PTEN*-Altered Subpopulations



Censoring times are shown as filled or open circles.
CI, confidence interval; OS, overall survival; PFS, progression-free survival.

SUPPLEMENTARY FIGURE 3. Alteration Landscape In Patients Analyzed by Next-Generation Sequencing, and Potential Role of Alternative Pathways on Progression-Free Survival



Graph shows progression-free survival of the 23 patients analyzed by next-generation sequencing. Each patient is labeled according to event type, and bars are shaded according to the source of archival tissue. Genes with at least two known/likely alterations within the cohort are listed below the graph – shading indicates the presence of a mutation (or change in copy number, where specified for *EGFR* and *KRAS*).

C, censored; D, death; PD, progressive disease.