Phase Ib/II trial of anti-CD33 monoclonal antibody BI 836858 and azacitidine in previously untreated older acute myeloid leukemia patients: Beat AML S2 sub-study results

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Table S1. Summary of Treatment-Emergent Adverse Events by Preferred Term in the Safety Analysis Population in Phase 2

Adverse Event MedDRA Preferred Term	All Patients (Total) (N = 36)
Treatment Emergent Adverse Events (TEAEs; Any Grade)	
Any TEAE - n (%)	36 (100.0)
Non-Hematologic (Incidence ≥30%)	
Nausea	21 (58.3)
Constipation	21 (58.3)
Decreased appetite	15 (41.7)
Fatigue	15 (41.7)
Hypophosphatemia	14 (38.9)
Diarrhea	12 (33.3)
Dyspnea	12 (33.3)
Blood creatinine increased	11 (30.6)
Hypoalbuminemia	11 (30.6)
Hematologic (Incidence ≥20%)	
Thrombocytopenia*	15 (41.7)
Neutropenia**	14 (38.9)
Anemia	15 (41.7)
Lymphopenia***	12 (33.3)
Leukopenia†	12 (33.3)
Febrile Neutropenia	8 (22.2)
Grade ≥3 TEAEs (Overall Incidence ≥10%)	
Any Grade ≥3 TEAE - n (%)	31 (86.1)
Neutropenia**	14 (38.9)
Thrombocytopenia**	14 (38.9)
Anemia	13 (36.1)
Leukopenia†	12 (33.3)
Lymphopenia***	11 (30.6)
Hypophosphatemia	11 (30.6)
Febrile Neutropenia	6 (16.7)
Hyponatremia	4 (11.1)
Hypotension	4 (11.1)

Cohort A = previously untreated patients aged ≥60 years with *TET2*, *IDH1*, *IDH2* or *WT1*-mutant AML; Cohort B = previously untreated patients aged ≥60 years with marker-negative AML.

MedDRA = Medical Dictionary for Regulatory Activities; AZA = Azacitidine.

All adverse events were graded using the Common Terminology Criteria for Adverse Events (CTCAE) version 4.03 and were coded using MedDRA version 19.1.

*Includes the MedDRA preferred terms thrombocytopenia and platelet count decreased.

**Includes the MedDRA preferred terms neutropenia and neutrophil count decreased.

***Includes the MedDRA preferred terms lymphopenia and lymphocyte count decreased.

†Includes the MedDRA preferred terms leukopenia and white blood cell count decreased.

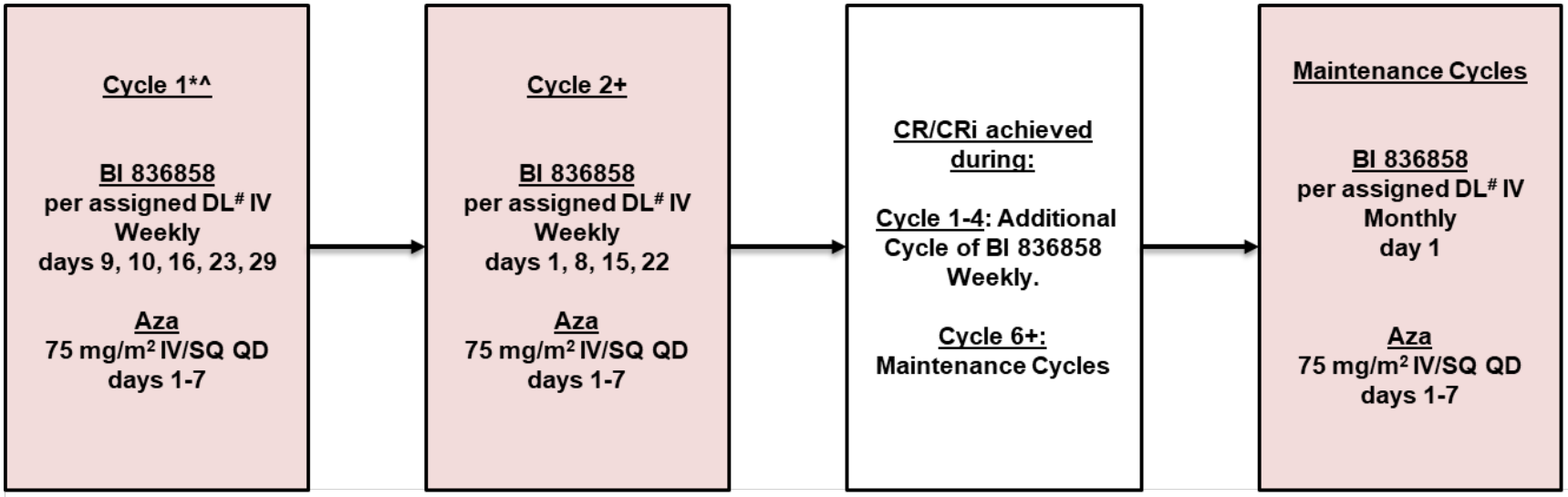
Note: Three (3) patients in the Phase 2 portion of the trial, 2 in Cohort A and 1 in Cohort B, were receiving ongoing study treatment at the time of the analysis.

Table S2. Patient Outcomes by ELN 2017 Risk Classification.

	ELN 2017 Favorable (n=12)	ELN 2017 Intermediate (n=19)	ELN 2017 Adverse (n=49)
Best Response, n (%)			
CR	4 (33.3)	5 (26.3)	10 (20.4)
CRi	3 (25.0)	3 (15.8)	4 (8.2)
MLFS	2 (16.7)	0 (0)	3 (6.1)
Partial Remission	1 (8.3)	0 (0)	1 (2.0)
Stable Disease	2 (16.7)	7 (36.8)	22 (44.9)
Treatment Failure - Death	0 (0)	1 (5.3)	1 (2.0)
Treatment Failure – Primary refractory	0 (0)	1 (5.3)	0 (0)
Unknown	0 (0)	2 (10.5)	8 (16.3)
Overall survival [months], median (95% CI)	18.2 (11.4-18.6)	11.3 (4.4-NR)	15.1 (8.1-20.6)

Patient outcomes are displayed for the combined Phase 1b and 2 portions of the study stratified by ELN 2017 risk classification.

Figure S1: Treatment Schema.



*Response evaluated days 22-29 on cycle 1, 2, and every other cycle until CR. After CR, response evaluated as needed.
^Next cycle is initiated when neutrophils ≥500/uL and platelets ≥20,000/uL or meet requirements for dose reduction.
#Dose levels: DL1=20 mg, DL2=40 mg, DL3=80 mg, DL4=160 mg.

Note that in cycle 1, 20 mg is administered on Day 9 followed by the remainder of the assigned dose on Day 10. The full dose is administered on Day 16 and weekly thereafter.