

Supplementary Table S1: Detailed information regarding patients' characteristics. AML: acute myeloid leukemia; BM: bone marrow; HSCT: hematopoietic stem cell transplantation; MDS: myelodysplastic syndrome; Refr: refractory; Rel: relapsed; T-: post-cytotoxic therapy; MDS-EB: MDS with excess of blasts; BM: bone marrow; N/A: not available

ID	ID EWOG	Sex	Diagnosis	Age at diagnosis	MDS ICC	Germline variants	Blasts at diagnosis (% in BM)	Cytogenetics ‡	Molecular biology ‡	Previous antineoplastic therapies	Previous allogenic HSCT †	% blasts at the time of venetoclax start	Extramedullary disease at the time of venetoclax start
1		M	Refr AML	10.5		no	71	Complex, t(9;22)	FLT3-ITD; BCR-ABL1p210	yes	no	80%	no
2		M	Refr AML	5.1		no	20	Normal	-	yes	no	5%	no
3		M	Refr AML	13.1		no	N/A	Del(3)	FLT3-ITD	yes	no	35%	no
4		M	T-MDS/AML	7.7		no	60	t(11;17)	MLL rearrangement	no	no	60%	no
5		F	MDS	15.5	MDS-EB	LIG4 and SH2B3 heterozygosis	6	-7	RIT1, EZH2, SETBP1, ASXL1, ETV6	no	no	30%	no
6		F	Rel AML	8.3		no	45	Normal	-	yes	yes (1)	45%	no
7		F	Rel AML	10.2		no	26%	normal	-	yes	no	26%	no
8		F	T- MDS/AML	5.2		SDHC heterozygosis	30	t(9;11)	MLL-AF9	yes	no	30%	no
9		F	T- MDS/AML	1.3		no	13	Hyper diploid, t(4;11)	MLL-AF1	yes	no	0%	no
10 ¶	D1223	M	MDS	14	MDS-EB	no	10	Del(7q)	-	yes	yes (2)	N/A	no
11	D1471	M	T-MDS/AML	10.8		no	10	-7	-	yes	no	14%	no
12		F	Refr AML	13.4		no	27	-7; t(3;3)	-	yes	no	20%	no
13	D1481	M	T-MDS/AML	10.1		no	25	Del(3q)	PTPN11, WT1	yes	no	20%	no
14		M	Refr AML	16.3		no	N/A	Complex	-	yes	no	0%	no
15		F	Rel AML	17.4		no	0%	t(6;11)	MLL-AFDN	yes	yes (1)	0%	yes (skin disease)
16		F	T- MDS/AML	6.6		no	44	t(11;19)	MLL-ENL	yes	yes (1)	5%	no
17	D1478	F	T- MDS/AML	9.1		no	38	-7	TP53	yes	no	75%	no
18	D1538	M	T- MDS/AML	14.2		no	48	-7	CBL, KRAS, ASXL2	yes	no	48%	no
19		M	Rel AML	12		no	36	t(7;12)	ETV6	yes	yes (2)	0%	no
20	D1263	M	Rel AML	13.4		no	44	Normal	WT1	yes	yes (1)	44%	no
21		F	Rel AML	17		no	9	t(6;9)	FLT3-ITD,DEK-CAN	yes	yes (2)	9%	no
22	D1495	M	Refr AML	15		no	20	Normal	NF1-	yes	no	18%	no
23		M	Rel AML	2.9		no	60	t(9;11)	MLL-AF9	yes	yes (1)	60%	no
24	D1503	M	MDS	17	MDS-EB	no	20	Normal	FLT3-ITD, WT1	yes	no	56%	no
25	D1359	M	Rel AML	5.7		no	55	Normal	CEBPA, WT1, KRAS, SMC1A	yes	yes (2)	55%	no
26		M	Rel AML	8.8		ATM and BRCA1 heterozygosis	10	Normal	-	yes	no	9%	no
27		F	Rel AML	2.4		no	10	t(16;16)	CBFA2T3-Glis2	yes	yes (1)	10%	no
28		F	Refr AML	1.7		no	80	t(16;16)	CBFA2T3-Glis2	yes	yes (1)	4%	yes (mastoid)
29	D1272	M	MDS	14.3	MDS-EB	no	5	N/A	WT1, FLT3-ITD	yes	yes (1)	48%	no
30		M	Rel AML	8.3		no	14	t(10;11)	MLL-AF10	yes	yes (1)	14%	no
31		F	T-MDS/AML	6.6		no	95	t(9;11)	MLL-AF9	yes	no	5%	no

¶ Patient 10 was included in a previous publication: Raedler J, Heyde S, Kolokythas M, Eichinger A, Binder V, Schmid I, Klein C, Feuchtinger T, Albert MH. Venetoclax and decitabine for relapsed paediatric myelodysplastic syndrome-related acute myeloid leukemia with complex aberrant karyotype after second stem cell transplantation. *Br J Haematol.* 2020 Jun;189(6):e251-e254. doi: 10.1111/bjh.16682. Epub 2020 Apr 28. PMID: 32342493.

† In parentheses the number of previous allogenic HSCT received

‡ cytogenetic and molecular aberrations were reported if present at the time of venetoclax start

Supplementary Table S2: Detailed information regarding venetoclax-based treatments and responses. CR: complete response; CRi: CR with incomplete count recovery; NR: non-response; PR: partial response; TF: treatment failure; ara-c: cytarabine; fluda: fludarabine; MRD: minimal residual disease; FCM-: flow cytometry; PCR-: polymerase chain reaction

ID	N cycles	N monotherapy	N azacitidine	N ara-c	N fluda + ara-c	N decitabine	N fluda + ara-C + idarubicine	N others	best response at any time	response to venetoclax therapy	days of therapy	dose (mg)	dose (mg/m2/die)
1	1	0	0	0	0	0	1	0	PR	PR	14	200	200
2	2	0	0	0	1	0	1	0	CR	FCM-MRD neg	14	200	266
3	1	0	0	0	0	0	2	0	NR	NR	11	200	120
4	2	0	0	0	0	0	2	0	CR	CRi	14	300	N/A
5	1	0	0	0	1	0	0	0	CR	FCM-MRD neg	18	400	240
6	2	0	0	0	2	0	0	0	CR	CRi	17	200	208
7	2	0	0	0	1	0	0	1 (fluda + araC + lipos doxo)	CR	CRi	14	400 (1.66)	240
8	2	0	0	0	0	0	2	0	CR	CRi	28	100	166
9	1	0	0	0	0	0	0	1 (ara-C+idarubicine)	NR	NR	28	200-400	360
10	6	1	0	0	0	5	0	0	CR	morphological; CR after 2 cycles maintained for 10 months; subsequent relapse, received other 3 cycles with PR then HSCT	28	20-50-100-200-400 mg	360
11	15	0	15	0	0	0	0	0	PR	PR (stable disease); after 15 cycles BM 15% blasts, received AML induction therapy	28	170	360
12	1	0	1	0	0	0	0	0	NR	NR	28	400	307
13	1	0	1	0	0	0	0	0	NR	NR	28	N/A	360
14	2	0	2	0	0	0	0	0	CR	FCM-MRD neg	28	N/A	N/A
15	7	0	7	0	0	0	0	0	CR	PCR-MRD neg (medullary); extramedullary CR (PET-FDG and skin biopsy neg after 7 cycles)	28	400	307
16	5	0	5	0	0	0	0	0	PR	PR	24	N/A	270
17	1	0	1	0	0	0	0	0	PR	PR	28	170	360
18	2	0	2	0	0	0	0	0	CR	morphological	28	250	360
19	2	0	1	0	0	1	0	0	NR	NR	28	N/A	200
20	2	0	2	0	0	0	0	0	CR	morphological	28	400-100	360
21	3	0	3	0	0	0	0	0	PR	PR	28	400	N/A
22	1	0	1	0	0	0	0	0	CR	CRi	28	N/A	360
23	2	0	2	0	0	0	0	0	PR	PR after 1 cycle; received a 2nd cycle with NR	22	200	344
24	1	0	1	0	0	0	0	0	NR	NR	26	400	360
25	4	1	3	0	0	0	0	0	NR	NR	28	170	360
26	1	0	1	0	0	0	0	0	CR	morphological	16	200	217
27	1	0	1	0	0	0	0	0	NR	NR	21	200	500
28	1	0	1	0	0	0	0	0	TF	TF for severe pancytopenia	9	250	N/A
29	2	0	1	1	0	0	0	0	CR	morphological	28	400	360
30	4	1	2	0	1	0	0	0	CR	FCM-MRD neg	28	N/A	360
31	2	0	1	0	1	0	0	0	CR	PCR-MRD neg	28	300	N/A

Supplementary Table S3: Detailed information regarding hematopoietic stem cell transplantations and outcomes. HSCT: hematopoietic stem cell transplantation; MUD: matched unrelated donor; MMUD: mismatch unrelated donor; haplo: haploidentical; TT: thiotepa; Bu: busulfan; Mel: melphalan; Cy: cyclophosphamide; VP16, etoposide; Flu, fludarabine; TBI: total body irradiation; Treo: treosulfan; CC: complete chimerism; MC: mixed chimerism; TRM: transplant-related mortality; IFI: invasive fungal infection; N/A: not available

ID	HSCT	Venetoclax therapy as bridge to HSCT	Conditioning	Donor	Last chimerism	Follow-up after transplant (months)	dead/alive at the end of follow-up	state of disease (if live)	Cause of death
1	yes	yes	TBI-Mel	MMUD 9/10	CC	21.0	alive	absent	
2	yes	yes	Bu-Cy-Mel	sibling	CC	5.3	alive	absent	
3	yes	no	TBI-TT-Mel	haplo	CC	4.4	alive	absent	
4	yes	yes	Bu-Cy-Mel	MMUD 9/10	CC	4.1	alive	absent	
5	yes	yes	TBI-Mel	haplo	CC	5.5	alive	absent	
6	yes	yes	TBI-VP16	haplo	CC	1.7	alive	absent	
7	yes	yes	Bu-Cy-Mel	MMUD 9/10	CC	1.6	alive	absent	
8	yes	yes	TBI-VP16	MUD	CC	1.2	alive	absent	
9	no	no					dead		disease progression
10	yes	yes	Flu-Cy-Mel	Haplo	N/A	1.1	dead		TRM
11	yes	yes	Bu-Cy-Mel	MUD	N/A	6.9	alive	absent	
12	yes	no	TBI-Mel	sibling	N/A	16.5	dead		disease progression
13	yes	yes	Bu-Cy-Mel	MUD	N/A	12.1	alive	absent	
14	yes	yes	Bu-TT-Flu	haplo	CC	9.3	alive	absent	
15	no	no					alive	absent	
16	no	no					dead		disease progression
17	yes	yes	Flu-Mel-TT	Haplo	MC	7.4	alive	absent	
18	yes	yes	Flu-Bu	MUD	N/A	6.0	alive	absent	
19	no	no					dead		disease progression and IFI
20	yes	yes	TT-Treo-Flu	MUD	CC	4.9	dead		TRM
21	no	no					dead	present	disease progression
22	yes	yes	Flu-Bu	MUD	N/A	3.4	alive	absent	
23	no	no					dead		disease progression and IFI
24	yes	yes	Bu-Cy-Mel	MUD	CC	2.8	dead		TRM
25	no	no					dead		disease progression
26	yes	yes	TBI-TT-Mel	sibling	CC	1.7	alive	absent	
27	no	no					dead		disease progression
28	no	no					dead		disease progression
29	yes	yes	TBI-Flu	MUD	MC	5.9	dead		relapse post HSCT
30	yes	yes	Bu-Cy-Mel	haplo	CC	2.5	alive	absent	
31	yes	yes	Bu-Cy-Mel	MUD	N/A	2.3	alive	absent	