

Table 1. Patient characteristics.

Case number/ sex	AaM, months	AaDx, months	AaV, months	Organs involved	Previous therapy	Vemurafenib		Concomitant therapy	Attempted to stop vemurafenib treatment	Status	Vemurafenib treatment, months
						Starting dose, mg/kg/day	Current dose mg/kg/day				
1/M	2	10	13	L, S, BM, Bo	VBL+PRED	48	12.3	VBL + PRED+ MTX + 6-MP; LD Ara-C + LD 2-CdA	Yes	Alive, receiving combined therapy	39.5
2/M	2	7	17	L, S, BM, Sk	VBL+PRED, ARA-C + 2-CdA	39.7	None	None	No	Dead (sepsis)	2.4
3/M	3	5	22	L, S, BM, LN, Sk	VBL+PRED, ARA-C + 2-CdA	34.8	13.3	LD Ara-C + LD 2-CdA	No	Alive, receiving vemurafenib monotherapy	45
4/M	1	6	18	L, S, BM, LN, Sk	VBL+PRED	41.7	12.9	VBL+PRED+MTX+6-MP	Yes	Alive with relapsed disease, receiving vemurafenib	29
5/F	1	22	26	L, S, BM, Sk, Bo, Lu	VBL+PRED, ARA-C + 2-CdA	51.6	15.8	VBL+PRED+MTX+6-MP; LD Ara-C + LD 2-CdA	Yes	Alive with skin-only relapsed disease, receiving vemurafenib monotherapy	30
6/F	9	22	24	L, S, BM, DI, Bo, Sk	VBL+PRED	44.4	None	LD Ara-C + LD 2-CdA	No	Dead (toxicity)	2.6

7/M	4	17	18	L, S, BM, Sk	VBL+PRED	47	17.4	LD Ara-C + LD 2-CdA	Yes	Alive, receiving vemurafenib monotherapy	35.8
8/F	2	13	14	L, S, BM, Sk	VBL+PRED	48	16.3	LD Ara-C + LD 2-CdA	Yes	Alive, receiving vemurafenib	34.3
9/M	1	13	23	L, S, BM, Sk, Bo	VBL+PRED, ARA-C + 2-CdA	40	13.0	None	No	Alive, receiving vemurafenib monotherapy	36.5
10/M	2	5	21	L, S, BM, Sk	VBL+PRED+MTX	40	None	LD Ara-C + LD 2-CdA	Yes	Alive with partial response, no therapy	3.9
11/F	10	19	40	L, S, BM, Sk	VBL+PRED, ARA-C + 2-CdA	14	7.5	None	No	Alive, receiving vemurafenib	17.9
12/M	2	7	23	Sk, Bo, LN	VBL+PRED, ARA-C + 2-CdA	21.8	20	None	No	Alive, receiving vemurafenib	19.1
13/M	28	31	62	Sk, Bo, lacrimal glands	VBL+PRED+MTX, mono 2-CdA	11	10.9	None	No	Alive, receiving vemurafenib monotherapy	17.4
14/M	5	13	59	Sk, Bo, CNS	VBL+PRED+MTX+6-MP, ARA-C + 2-	16	11.5	None	No	Alive, receiving vemurafenib	29.3
15/F	9	18	18	L, S, BM, Sk	VBL+PRED+6-MP	24.7	17	VBL+PRED+MTX+6-MP	No	Alive, receiving vemurafenib	24.9

AaM: age at manifestation, AaDx: age at diagnosis, AaV: age at the start of vemurafenib treatment, L: liver, S: spleen, BM: bone marrow, Bo: bones, Lu: lungs, LN: lymph nodes, CNS: central nervous system, DI: diabetes insipidus, Sk: skin, LD Ara-C: cytosine-arabioside at 100 mg/m² every 12 h for 5 days, Ara-C: cytosine-arabioside at 500 mg/m² every 12 h for 5 days, LD 2-Cda: cladribine at 6 mg/m²/day for 5 days, 2-CdA: cladribine at 9 mg/m²/day for 5 days.