Table S1. Patient characteristics and clinical response to DC vaccination, grouped according to the WT1 construct used. Responders to WT1/DC vaccination: MR, molecular remission (normalization of *WT1* mRNA levels in blood and/or bone marrow); SD, stable disease (stabilization of increased *WT1* mRNA levels - the duration of SD period is indicated between parentheses); NR, non-responders.

UPN	Sex/Age/Cytog. risk	Number of DCs (x10^6/dose)	Response category	OS / Disease status
01	F/77/int	5	MR	Died
06	M/41/int	10	MR	119.3 mo +/ CR1, alive
08	M/33/int	20	PR → CR & MR	116.8 mo +/ CR1, alive
10	M/53/fav (inv(16))	20	MR	109.4 mo +/ CR1, alive
16	F/59/int (t(1;5))	10	PR → CR & MR	Died
02, 03, 05, 07, 09	*Respectively*: F/78/int, M/67/adv (complex), F/39/int, M/63/fav (t8;21), M/82/int	*Respectively*: 5, 10, 5, 20, 5	NR (all)	Died (all)

UPN	Sex/ Age	WHO type (FAB)/ Cytog. risk	Unfavorable prognostic features	Pre-DC treatment	Status pre-DCs	Time Dx to start DCs	Response category	Treatment post-4th DC vaccination	OS / Disease status
11*	M/62	AML-mds/ int	• Age >60 • MDS phase pre-AML • WT1 mRNA ↑ (post-C)	I: MICE C: ICE (1x)	CR1	8.0 mo	MR	Additional DC vaccines; R1 (51.6 mo post-start WT1/DCs): supportive care	54.0 mo / R1, died
12*	F/68	AML-mds/ int	• Age >60 • MDS phase pre-AML • WT1 mRNA ↑ (post-C)	I: MICE C: ICE (2x)	CR1	7.7 mo	NR	R1 (5.0 mo post-start WT1/DCs): HAM (1x); CR2 (6.5 mo post-start WT1/DCs)	99.3 mo + / CR2, alive
15	F/77	AML-t(8;21)/ fav	• Age >60 • WT1 mRNA ↑ (post-C)	I: MICE RI: HAM C: HAM	CR1	6.3 mo	MR	Additional WT1/DC vaccines	93.1 mo + / CR1, alive
46	F/36	AML-nos (M1)/int	• Hyperleukocytosis (WBC 100.10^9/L) • WT1 mRNA ↑ (post-C) • No sib donor for allo-HSCT	I: DCE C: DC (2x)	CR1	7.7 mo	NR	R1 (1.4 mo post-start WT1/DCs) : I: HAM; PR; RI: HAM; CR2; MUD allo-HSCT	5.2 mo / CR2, died (transplant-related VOD)
47	M/61	AML-nos (M5a)/int	• Age >60 • WT1 mRNA ↑ (post-C)	I: ICE C: ICE (2x)	CR1	7.4 mo	NR	R1 (3.9 mo post-start WT1/DCs): I & C: HAM; CR2; MUD allo-HSCT	9.5 mo / CR2, died (GVHD)
48	M/74	AML-nos (M1) /int	• Age >60 • WT1 mRNA ↑ (post-C)	I: DC C: C	CR1	10.6 mo	SD (3.0 mo)	R1 (4.4 mo post-start WT1/DCs): decitabine (4 cycles); CR2; decitabine (1 cycle); R2 (2.5 mo after CR2; 11 mo post-start WT1/DC): decitabine	14.0 mo / R2,died

Table S1A. Patient characteristics and clinical response to vaccination with DCs electroporated with mRNA derived from *WT1* construct 1 ("WT1 group"; for details of this construct, see Figure 1). The first 10 patients described in the upper panel (responders UPN01, 06, 08, 10, 16; non-responders UPN02, 03, 05, 07, 09) were enrolled in NCT0083400211; all other patients in NCT00965224. If not indicated in the table, the patients received 10 x 10^6 DCs/dose.

UPN	Sex/ Age	WHO type (FAB)/ Cytog. risk	Unfavorable prognostic features	Pre-DC treatment	Status pre-DCs	Time Dx to start DCs	Response category	Treatment post-4th DC vaccination	OS / Disease status
13*	M/69	AML-nos (M5b)/int (+11)	• Age >60 • WT1 mRNA ↑ (post-C)	I: MICE C: ICE (2x)	CR1	8.2 mo	NR	R1 (4.1 mo post-start WT1/DCs): HAM; no remission; sib allo-HSCT; CR2	11.0 mo / CR2, died (GVHD)
14	M/50	AML-nos (M2)/int	• *FLT3-ITD* mutation • WT1 mRNA ↑ (post-I and post-C) • No sib donor for allo-HSCT	I: ICE C: DC (2x)	CR1	7.7 mo	MR	Additional WT1/DC vaccines	94.0 mo + / CR1, alive
17	F/42	AML-t(8;21)/ fav	• WT1 mRNA ↑ (post-I and post-C) • No sib donor for allo-HSCT	I: DCE C: DC (2x)	CR1	8.2 mo	MR	Additional WT1/DC vaccines; R1 (9.7 mo post-start WT1/DCs): HAM (2x); CR2; MUD allo-HSCT	89.2 mo + / CR2, alive
20	F/68	AML-mds/ int	• Age >60 • MPN (PV) phase pre-AML • PR (10% blasts in bone marrow) • WT1 mRNA ↑ (post-C)	I: ICE RI: HAM	PR	4 mo	NR	Increasing number of blasts in blood from 1.0 mo post-start WT1/DCs; supportive care	3.5 mo / PD, died
21	M/77	AML-mds/ int	• Age >60 • MDS phase pre-AML • WT1 mRNA ↑ (post-I)	I: MICE C: ICE	CR1	5.8 mo	SD (11.0 mo)	Additional WT1/DC vaccines; R1 (31.9 mo post-start WT1/DCs); supportive care	44.3 mo / PD, died
22	M/55	AML-nos (M6a)/int	• Erythroleukemia • No sib donor for allo-HSCT	I: DCE C: DC (2x)	CR1	6.6 mo	Undefinable	Additional WT1/DC vaccines	79.8 mo + / CR1, alive

Table S1B. Patient characteristics and clinical response to vaccination with DCs electroporated with mRNA derived from *WT1* construct 2 ("WT1-DC-LAMP group"; for details of the construct, see Figure 1).

UPN	Sex/ Age	WHO (FAB)/ Cytog. risk	Unfavorable prognostic features	Pre-DC treatment	Status pre-DCs	Time Dx to start DCs	Response category	Treatment post-4th DC vaccination	OS / Disease status
28	F/43	AML-inv(16)/ fav	• Hyperleukocytosis (WBC 169.10^9/L) • WT1 mRNA ↑ (post-C) • No sib donor for allo-HSCT	I: DCE C: DC (2x)	CR1	6.8 mo	NR	Additional WT1/DC vaccines; R1 (2.0 mo post-start WT1/DCs): HAM (1x); CR2; MUD allo-SCT	77.0 mo + / CR2, alive
29	F/46	AML-nos (M1)/int	• No sib donor for allo-HSCT	I: DCE C: DC (2x)	CR1	7.7 mo	NR	Additional WT1/DC vaccines; R1 (59.8 mo post-start WT1/DCs); HAM, no CR, clofarabine, then MUD allo-SCT	62.3 mo / R1, died (respiratory failure, lung aspergillosis)
30	F/72	AML-nos (M2)/int	• Age >60	I: MICE C: ICE	CR1	5.4 mo	NR	Additional WT1/DC vaccines; R1 (7.6 mo post-start WT1/DCs): HAM (1x); CR2 (CRi); R2 (7.5 mo after CR2; 17.2 mo post-start WT1/DCs): supportive care	17.9 mo / R2, died
33	F/72	AML-nos (M2)/int (+4)	• Age >60 • WT1 mRNA ↑ (post-C)	I: MICE C: ICE (2x)	CR1	6.9 mo	SD (12.1 mo)	Additional WT1/DC vaccines; R1 (21.6 mo post-start WT1/DCs) : thioguanine	39.2 mo / PD, died
34	M/48	AML-inv(16)/ fav	• *FLT3-ITD* mutation at diagnosis • Previous relapse • No sib donor for allo-HSCT	I: 7 cycles of FLAG, 4 of them with GO; CR1; R1: RI: CIA	CR2	4.3 mo post-R1; (18.7 mo post-1st diagnosis)	NR	Additional WT1/DC vaccines; R2 (16.2 mo post-start WT1/DCs):RI: MEC; CR3 (19 mo post-start WT1/DCs); C: C; maintenance with 5-azacitidine	68.2 mo + / CR3, alive
35	M/72	AML- nos (M5b)/int	• Age >60	I: MICE C: ICE (2x)	CR1	7.1 mo	SD** (5.1 mo)	Additional WT1/DC vaccines; R1 (40.2 mo post-start WT1/DCs; increasing number of blasts in blood from 49.0 mo post-start WT1/DCs): HAM (1x); CR2 (51.8 mo post-start WT1/DCs); R2 (59.8 mo post-start WT1/DCs); mitoxantrone (1x), 1 WT1/DC vaccine, decitabine (2 cycles): no CR; RI: HAM (1x); CR3 (CRi, 67.6 mo post-start WT1/DCs); C: C	68.7 mo + / CR3, alive
36	F/66	AML-nos (M5)/int	• Age >60 • *FLT3-ITD* mutation • WT1 mRNA ↑ (post-C)	I: MICE C: ICE (2x)	CR1	6.6 mo	NR	Only 3 WT1/DC vaccines administered; R1 (0.9 mo post-start WT1/DCs) : HAM (2x); CR2; MUD allo-HSCT; R2 (12.2 mo post-start WT1/DCs): 2nd MUD allo-HSCT; CR3; R3 (24 mo post-start DCs): thioguanine	25.6 mo / R3, died
38	F/73	AML-nos (M0)/int	• Age >60 • WT1 mRNA ↑ (post-I and post-C)	I: MICE RI: HAM C: HAM	CR1	7.3 mo	NR	Additional WT1/DC vaccines; R1 (2.1 mo post-start WT1/DCs): supportive care	13.8 mo / R1, died

Table S1C. Patient characteristics and clinical response to vaccination with DCs electroporated with mRNA derived from *WT1* construct 3 ("WT1-DC-LAMP-OPT group"; for details of the construct, see Figure 1).

Abbreviations and clarifications, listed according to the columns

UPN, unique patient number; *, the DCs were cultured without keyhole limpet hemocyanin (KLH). **Sex / Age / Cytog. risk**: age at start of WT1/DC vaccination; F, female; M, male; Cytog. risk, cytogenetic risk group (int, intermediate; fav, favorable; adv: adverse; the cytogenetic abnormality found is indicated between parentheses (inv, inversion; t, translocation)). **WHO (FAB) /Cytog. risk**: WHO, World Health Organization classification for AML; FAB, French-American-British classification for AML (indicated for AML not otherwise specified (AML-nos)); AML-mds, AML with myelodysplasia-related changes; AML-nos, AML not otherwise specified; AML-t(8;21), AML with t(8;21) recurrent genetic abnormality; AML-inv(16), AML with inv(16) recurrent genetic abnormality; Cytog. risk: cytogenetic risk group (int, intermediate; fav, favorable; the cytogenetic abnormality is indicated between parentheses for the intermediate risk group, in case the bone marrow karyotype was not normal). **Unfavorable prognostic features**: MDS, myelodysplastic syndrome; ↑, increased above background; post-C, after consolidation chemotherapy; PR, partial hematological remission; post-I, after induction chemotherapy; ITD, internal tandem duplication; hyperleukocytosis, at diagnosis; WBC, white blood cell count; MPN (PV), myeloproliferative neoplasia (polycythemia vera). **Pre-DC treatment**: I: MICE, induction with mitoxantrone + cytarabine + etoposide; C: ICE, consolidation with idarubicin + cytarabine + etoposide; RI: HAM, reinduction with high-dose cytarabine + mitoxantrone; C: HAM, consolidation with HAM; I: DCE, induction with daunorubicin + cytarabine + etoposide; C: DC, consolidation with daunorubicin + cytarabine; I: DC, induction with DC; C: C, consolidation with cytarabine; I: FLAG, fludarabine + cytarabine; GO, gemtuzumab ozogamicin; RI: CIA, reinduction after 1st early relapse with clofarabine + idarubicin + cytarabine; RI: MEC, reinduction after 2nd relapse with mitoxantrone + etoposide + cytarabine. **Status pre-DC**: CR1, 1st complete remission; PR, partial remission; CR2, 2nd CR. **Time Dx to start DCs**: time from diagnosis to the start of DC vaccination (mo, months). **Response category**: MR, molecular remission; PR→ CR, conversion of partial into complete hematological remission by WT1/DCs alone; NR, non-responder; SD, stable disease; **patient UPN35 showed 5% blasts in the bone marrow 40.2 mo after the start of DC vaccination and relapsed molecularly (with an increase above background of blood *WT1* transcript levels), while he was on bimonthly DC vaccination with the *WT1-DC-LAMP-OPT* construct. The rhythm of DC vaccinations was increased to biweekly, upon which the patient had to undergo a new apheresis for the production of new DC vaccines, which were electroporated with the wild type *WT1* construct. The SD phase occurred during the biweekly injection of both types of vaccines (2 months for the *WT1-DC-LAMP-OPT* construct and 3 months for the *WT1* construct). Control marrow examinations showed 4.6% and 14.6% blasts, respectively, 45.3 mo and 49 mo after the start of DC vaccination and, respectively, 3.6 mo after the start and 2.3 mo after the end of the SD period (which lasted 5.1 mo). **Treatment post-4th DC vaccination**: R, relapse (R1, 1st; R2, 2nd; R3, 3rd); allo-HSCT, allogeneic hematopoietic stem cell transplantation; MUD, matched unrelated donor; CRi, CR with incomplete blood count recovery. **OS / Disease status**: OS, overall survival (as of December 31, 2016) from the start of DC vaccination; PD, progressive disease; VOD, veno-occlusive disease; GVHD, graft-vs-host disease.

	% $WT1_{37-45}$ $tetramer^+$ $CD8^+$ T-cells		% $WT1_{126-134}$ $tetramer^+$ $CD8^+$ T-cells		% $WT1_{187-195}$ $tetramer^+$ $CD8^+$ T-cells		% $WT1_{235-243}$ $tetramer^+$ $CD8^+$ T-cells	
UPN	**Pre**	**Post**	**Pre**	**Post**	**Pre**	**Post**	**Pre**	**Post**
UPN17	0.190	0.160	0.370	0.190	0.210	0.140	0.420	0.380
UPN28	0.150	0.140	0.100	0.110	0.140	0.110	NE	NE
UPN30	0.043	0.084	0.058	0.119	0.033	0.069	NE	NE
UPN34	0.026	0.044	0.068	0.074	0.040	0.044	NE	NE
UPN35*	0.019	0.090	0.025	0.139	0.020	0.029	NE	NE
UPN38	0.101	0.056	0.122	0.068	0.107	0.069	NE	NE
UPN47	0.010	0.006	0.055	0.018	0.057	0.590	0.126	0.034

UPN, unique patient number; Pre, pre-DC vaccination; Post, post-4th DC vaccination; *, long-term responder; the values underlined represent a >1.5-fold increase in tetramer+ CD8+ T-cells; NE, not evaluable or not done. The values for long-term responders UPN01 and UPN08 and non-(long-term) responders UPN05, UPN09 and UPN16 can be found in reference 17; these data were included in the statistical evaluation of the correlation between long-term clinical response and poly-epitope WT1-specific tetramer+ CD8+ T-cells.

Table S2. Pre- and post-DC vaccination WT1-specific $tetramer^+$ $CD8^+$ T-cell frequencies in the blood of HLA-A*0201$^+$ AML patients.