

Table S1. Demographics and disease characteristics of infants enrolled in AAML03P1 or AAML0531, combined

Characteristic (if known)	All Infants		Infants Age 0–179 d		Infants Age 180–364 d		P
	N	%	N	%	N	%	
Study							
AAML03P1	39	27%	21	33%	18	23%	0.196
AAML0531	103	73%	43	67%	60	77%	
Gender							
Male	70	49%	32	50%	38	49%	0.879
Female	72	51%	32	50%	40	51%	
Race							
American Indian or Alaskan Native	0	0%	0	0%	0	0%	1.000
Asian	1	1%	0	0%	1	1%	1.000
Pacific Islander	0	0%	0	0%	0	0%	1.000
Black or African American	14	10%	5	8%	9	12%	0.500
White	112	79%	51	80%	61	78%	0.428
Other or unknown	15	11%	8	13%	7	9%	1.000
FAB group*							
FAB M0	5	4%	1	2%	4	6%	0.379
FAB M1	3	3%	1	2%	2	3%	1.000
FAB M2	8	7%	4	8%	4	6%	1.000
FAB M4	19	17%	7	14%	12	19%	0.439
FAB M5	54	48%	26	52%	28	45%	0.564
FAB M6	1	1%	1	2%	0	0%	0.451
FAB M7	22	20%	10	20%	12	19%	0.969
Cytogenetic group							
Normal	12	8%	5	8%	7	9%	0.859
t(8;21)	0	0%	0	0%	0	0%	1.000
inv(16)	4	3%	1	2%	3	4%	0.630
11q23	70	49%	34	53%	36	46%	0.260
t(6;9)	0	0%	0	0%	0	0%	1.000
Monosomy 7	1	1%	0	0%	1	1%	1.000
Del(7q)	6	4%	2	3%	4	5%	1.000
Monosomy 5/del5q	1	1%	0	0%	1	1%	1.000
Trisomy 8	6	4%	1	2%	5	6%	0.228
Other	27	19%	13	20%	14	18%	0.633
Unknown	15	11%	8	13%	7	9%	
FLT3 ITD							
ITD negative	113	100%	50	100%	63	100%	1.000
ITD positive	0	0%	0	0%	0	0%	
Unknown	29		14		15		
12p/ETV6 mutation							
No	132	93%	54	96%	67	94%	0.691
Yes	6	4%	2	4%	4	6%	
CNS disease							
CNS1	83	60%	38	61%	45	59%	0.804
CNS2 + CNS3	55	40%	24	39%	31	41%	
Unknown	4		2		2		
WBC count at diagnosis							
WBC ≤ 100,000	102	72%	44	69%	58	74%	0.460
WBC > 100,000	40	28%	20	31%	20	26%	
Risk group (AAML0531 definition)							
Intermediate	136	96%	63	98%	73	94%	0.223
Low	4	3%	1	2%	3	4%	0.627
High	2	1%	0	0%	2	3%	0.223

FAB indicates French-American-British; ITD, internal tandem duplication; CNS, central nervous system; WBC, white blood cell.

*FAB type was available for 112 patients.

Table S2. Univariable analyses for EFS and OS from study entry for infants (N = 142)

Category	EFS from study entry				OS from study entry			
	N	HR	95% CI	P	N	HR	95% CI	P
Age group								
0–179 d	64	0.935	0.6–1.46	0.768	64	0.776	0.45–1.35	0.367
180–364 d	78	1			78	1		
Treatment								
noGO	64	1			64	1		
GO	78	0.747	0.48–1.16	0.193	78	0.714	0.42–1.23	0.221
11q23 abnormality								
No	56	1			56	1		
Yes	73	1.238	0.77–1.99	0.377	73	0.980	0.54–1.77	0.946
WBC group								
Low ($\leq 100,000$)	102	1			102	1		
High ($> 100,000$)	40	1.501	0.94–2.39	0.086	40	1.218	0.68–2.17	0.504
12p /ETV6 abnormality								
No	121	1			121	1		
Yes	6	1.219	0.45–3.34	0.701	6	0.821	0.2–3.39	0.785
Risk group								
Low risk	4	1.074	0.34–3.41	0.904	4	–	–	0.986
Intermediate risk	136	1			136	1		
High risk	2	1.829	0.45–7.45	0.400	2	3.863	0.94–15.92	0.061
CNS category								
CNS1	83	1			83	1		
CNS2 + CNS3	55	1.533	0.98–2.41	0.064	55	1.023	0.58–1.81	0.939

EFS indicates event-free survival; OS, overall survival; HR, hazard ratio; CI, confidence interval; GO, gemtuzumab ozogamicin; WBC, white blood cell; CNS, central nervous system.

Table S3. Multivariable analyses for EFS and OS from study entry for infants (N = 116)

Category	EFS from study entry				OS from study entry			
	N	HR	95% CI	P	N	HR	95% CI	P
Age group								
0–179 d	51	0.931	0.55–1.57	0.788	51	1.058	0.53–2.1	0.872
Infant, 180–364 d	65	1			65	1		
Treatment								
noGO	57	1			57	1		
GO	59	0.676	0.4–1.15	0.148	59	0.502	0.25–1.02	0.057
11q23 abnormality								
No	45	1			45	1		
Yes	71	1.184	0.68–2.08	0.556	71	0.914	0.46–1.84	0.802
WBC group								
Low ($\leq 100,000$)	82	1			82	1		
High ($> 100,000$)	34	1.279	0.74–2.2	0.375	34	1.146	0.55–2.4	0.718
12/ETV6 abnormality								
No	110	1			110	1		
Yes	6	1.356	0.46–3.99	0.581	6	1.064	0.24–4.82	0.936
Risk group								
Low risk	4	1.010	0.29–3.52	0.988	4	–	–	
Intermediate risk	110	1			110	1		
High risk	2	2.655	0.56–12.62	0.220	2	7.141	1.3–39.38	0.024
CNS category								
CNS1	67	1			67	1		
CNS2 + CNS3	49	1.334	0.8–2.22	0.268	49	0.808	0.4–1.63	0.549

EFS indicates event-free survival; OS, overall survival; HR, hazard ratio; CI, confidence interval; GO, gemtuzumab ozogamicin; WBC, white blood cell; CNS, central nervous system.

Table S4. Toxicities in infants overall, during Induction I, and during Intensification II

	noGO (n = 64)		GO (n = 78)		
AAML03P1 + AAML0531					
	<i>N</i>	%	<i>N</i>	%	<i>P</i>
Liver VOD	3	5%	5	6%	0.730
Ind I	0	0%	2	3%	0.501
Int II	0	0%	0	0%	1.000
LVSD	8	13%	3	4%	0.055
Ind I	1	2%	1	1%	1.000
Int II	1	3%	0	0%	0.466
AAML0531 only					
	<i>N</i>	%	<i>N</i>	%	<i>P</i>
Grade 3–5 infection	41	64%	24	62%	0.797
Ind I	15	23%	11	28%	0.589
Int II	17	59%	11	58%	0.960
Sterile-site bacteria	36	56%	21	54%	0.812
Ind I	7	11%	6	15%	0.550
Int II	15	52%	9	47%	0.768
Sterile-site fungus	2	3%	3	8%	0.364
Ind I	2	3%	2	5%	0.633
Int II	0	0%	0	0%	1.000
AAML0531 only					
	Median (d)	Range (d)	Median (d)	Range (d)	<i>P</i>
ANC recovery Ind I	30	18–57	28	10–44	0.293
ANC recovery Int II	33	10–48	32	23–47	0.881
Platelet recovery Ind I	25	14–42	26	18–29	0.886
Platelet recovery Int II	27	11–56	30	17–40	0.711

GO indicates gemtuzumab ozogamicin; VOD, veno-occlusive disease; Ind I, Induction I; Int II, Intensification II; LVSD, left ventricular systolic dysfunction; ANC, absolute neutrophil count.