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Supplemental Tables for:
FDA Approval Summary: Mylotarg for Treatment of Patients with Relapsed or Refractory CD33-Positive Acute Myeloid Leukemia
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Table S1: Clinical Studies of GO Monotherapy in R/R AML

Study/Reference	Design	Population	Endpoints
Unfractionated			
Study 201	Single-arm, open-label Phase 2 trial • GO 9 mg/m^2 x 2-3, 14-28d apart	Adults with CD33+ AML in first relapse after CR ≥ 6 months - 84 patients	CR rate
Study 202	Single-arm, open-label Phase 2 trial • GO 9 mg/m^2 x 2-3, 14-28d apart	Adults with CD33+ AML in first relapse after CR ≥ 6 months - 95 patients	CR rate
Study 203	Single-arm, open-label Phase 2 trial • GO 9 mg/m^2 x 2-3, 14-28d apart	Adults ≥ 60 years with CD33+ AML in first relapse after CR ≥3 months - 98 patients	CR rate
Study 101	Single-arm, open-label Phase 1 dose-escalation trial • GO 0.25-9 mg/m^2 x 3, ≥14d apart	Adults with R/R CD33+ AML - 41 patients	Safety
Study 102	Single-arm, open-label Phase 1 dose-escalation trial • GO 6-9 mg/m^2 x1-2 doses, ≥14d apart (< 3 years, per kg dosing)	Pediatric patients with R/R CD33+ AML - 29 patients	CR+CRp rate
Study 103	Single-arm, open-label Phase 1-2 dose-escalation trial GO 6-9 mg/m^2 x 2, ≥14d apart	Japanese adults with R/R CD33+ AML - 40 patients	CR+CRp rate
Study 100374	Single-arm, open-label Phase 4 dose-escalation trial	Adults with relapsed CD33+ AML post HSCT - 37 patients	CR+CRp rate

	• GO 2-6 mg/m^2 x 2, ≥14d apart • Consolidation: up to 4 doses GO		
Study 100863	Single-arm, open-label Phase 4 trial • GO 9 mg/m^2 x 2, 14d apart • Studied steroid prophylaxis	Adults with R/R CD33+ AML - 23 patients	Safety
Roboz 2002 [33]	Prospective, single-arm, open-label trial • GO 9 mg/m^2 x 2, 14d apart	Adults with CD33+ R/R or untreated AML, CML-BC, or RAEB-T - 43 patients Children with CD33+ R/R AML - 15 patients	CR+CRp rate
Zwaan 2003 [17]	Retrospective, single-arm, open-label trial • GO 4-9 mg/m^2 x1-3 doses	Adults with CD33+ R/R AML - 24 patients	CR+CRp rate
Piccaluga 2004 [34]	Prospective, single-arm, open-label trial • GO 9 mg/m^2 x 2-3 q14-28d (n=16) or • GO 6 mg/m^2 x 2-3, q14-28d (n=7) or • GO 1.5 mg/m^2 x 2-3, q14-28d (n=1)	Adults with untreated or R/R AML - 38 patients	CR+CRp rate
van der Heiden 2006 [26]	Retrospective, open-label trial • GO 6-9 mg/m2 x1-3 doses	Children with CD33+ R/R AML - 5 patients (unfractionated)	CR+CRp rate
Brethon 2006 [10]	Retrospective, open-label trial • GO 7.5-9 mg/m^2 x 1-2,14-28d apart	Adults with R/R AML - 6 patients (unfractionated)	CR+CRp rate
Thomas 2005 [11]	Retrospective, open-label trial • GO 6 mg/m^2 x2, 14d apart		CR+CRp rate
Fractionated GO 3 mg/m^2 d 1, 4, 7			

Taksin 2007 [9] (MyloFrance 1)	Single-arm, open-label Phase 2 trial • GO 3 mg/m^2 d 1, 4, 7	Adults with CD33+ AML in first relapse after CR ≥3, ≤18 months - 57 patients	CR+CRp rate
Brethon 2006 [10]	Retrospective, open-label trial • GO 3 mg/m^2 d1, 4, 7	Children with CD33+ R/R AML - 6 patients (fractionated)	CR+CRp rate
Thomas 2005 [11]	Retrospective, open-label trial • GO 3 mg/m^2 d1, 4, 7	Adults with R/R AML - 24 patients (fractionated)	CR+CRp rate

Abbreviations: AML, acute myeloid leukemia; CD, cluster of differentiation; CML-BC, chronic myeloid leukemia in blast crisis; CR, complete remission; CrCl, creatinine clearance; CRp, complete remission with incomplete platelet recovery; d, days; GO, gemtuzumab ozogamicin; h, hours; HSCT, hematopoietic stem cell transplantation; RAEB-T, refractory anemia with excess blasts in transformation; R/R, relapsed and/or refractory.

Table S2: Early mortality by GO monotherapy dose-schedule for R/R AML

Dose regimen	Study	No. Patients	Early Deaths[a] N (%, 95% CI)
9 mg/m^2 x 2[b]	**All combined**	**410**	**70 (17%, 14-21%)**
	201	84	16 (19%)
	202	95	14 (15%)
	203	98	21 (21%)
	101	7	1 (14%)
	102	13	2 (15%)
	103	31	4 (13%)
	100863	23	5 (22%)
	Piccaluga, 2004 [34]	16	1 (6%)
	Roboz, 2002 [33]	43	6 (14%)
6 mg/m^2 x 2[b]	**All combined**	**41**	**5 (12%, 5-27%)**
	101	8	2 (25%)
	102	14	1 (7%)
	103	6	0 (0%)
	100374	6	0 (0%)
	Piccaluga, 2004 [34]	7	2 (29%)
3 mg/m^2 d1,4,7	**All combined**	**81**	**7 (9%, 4-18%)**
	MyloFrance 1	57	4 (7%)
	Thomas, 2005 [11]	24	3 (13%)[c]

Abbreviations: CI, confidence interval; No., number.

[a]Defined as through treatment day 43 for studies 101-203, 100863, 100374, MyloFrance 1, and Thomas 2005, "during the treatment period" in Piccaluga 2004, and as "early deaths" in Roboz 2002.

[b]Note that 201-203, 101, Piccaluga 2004 and Roboz 2002 allowed for up to 3 doses of GO.

[c]Determined through personal communication with Dr. Thomas.

Table S3: Time to hematopoietic recovery for CR/CRp responses

Parameter	Median time in days (range)	
	9 mg/m^2 x 2 201-203 (N=98)	3 mg/m^2 d1,4,7 MyloFrance-1 (N=19)
ANC > 0.5 Gi/L	42 (16-100)	23 (10-38)*
Platelets > 50 Gi/L	51 (15-528)†	20 (0-43)‡

Abbreviations: ANC, absolute neutrophil count; d, days; N, number.

*One patient had missing data.

†Given failure to achieve the target platelet level, 22 patients were censored at the date of last laboratory evaluation prior to HSCT, other anti-leukemic therapy, or death (whichever came first).

‡Given failure to achieve the target platelet level, 1 patient was censored on day 43. Two further patients had missing data.