

Supplementary

Supplemental Table 1. Study disposition for the overall population

Disposition, n (%)	Cohort 1 n = 69	Cohort 2 n = 81	Cohort 3 n = 60	Total N = 210
Completed	19 (27.5)	13 (16.0)	14 (23.3)	46 (21.9)
Discontinued	50 (72.5)	68 (84.0)	46 (76.7)	164 (78.1)
Adverse event	8 (11.6)	5 (6.2)	5 (8.3)	18 (8.6)
Bone marrow transplant	1 (1.4)	2 (2.5)	1 (1.7)	4 (1.9)
Clinical progression	3 (4.3)	1 (1.2)	1 (1.7)	5 (2.4)
Complete response	8 (11.6)	9 (11.1)	11 (18.3)	28 (13.3)
Lost to follow-up	1 (1.4)	2 (2.5)	0	3 (1.4)
Physician decision	3 (4.3)	6 (7.4)	1 (1.7)	10 (4.8)
Pregnancy	0 (0.0)	1 (1.2)	0 (0.0)	1 (0.5)
Progressive disease	23 (33.3)	37 (45.7)	26 (43.3)	86 (41.0)
Patient withdrawal	3 (4.3)	5 (6.2)	1 (1.7)	9 (4.3)

Supplemental Table 2. Summary of response duration, progression-free survival, and overall survival in patients who achieved complete response and partial response^a

Achieved CR				
	Cohort 1 n = 17	Cohort 2 n = 21	Cohort 3 n = 20	Total n = 58
DOCR				
Median, months	31.2	32.9	NR	NR
95% CI	13.6-NR	8.5-NR	16.8-NR	16.1-NR
Response \geq 4 years, %	43.1	43.0	71.4	51.6
PFS				
Median, months	30.8	56.5	NR	56.5
95% CI	16.3-NR	11.1-NR	21.7-NR	21.7-NR
5-year PFS rate, %	42.9	25.1	61.8	44.3
OS				
Median, months	NR	NR	NR	NR
95% CI	53.8-NR	NR-NR	NR-NR	NR-NR
5-year OS rate, %	74.3	83.8	88.9	82.8
Achieved PR				
	Cohort 1 n = 37	Cohort 2 n = 31	Cohort 3 n = 24	Total N = 92
DOPR				
Median, months	22.1	7.9	13.9	11.1
95% CI	8.4-29.2	4.9-11.8	5.5-24.4	8.2-16.8
Response \geq 3 years, %	6.5	N/A	N/A	3.0
PFS				
Median, months	22.2	11.3	19.7	13.8
95% CI	13.6-32.7	7.6-13.8	8.5-27.0	12.0-22.1
PFS rate at 3 years, %	11.1	NR	20.2	10.3

OS				
Median, months	NR	NR	NR	NR
95% CI	NR-NR	NR-NR	46.4-NR	NR-NR
5-year OS rate, %	78.9	75.6	69.7	75.5

CR, complete response; DOCR, duration of complete response; DOPR, duration of partial response; NR, not reached; OS, overall survival; PFS, progression-free survival; PR, partial response.

^aFrom product-limit (Kaplan-Meier) method for censored data.

Supplemental Table 3. Time to CR and duration of CR by pembrolizumab exposure

	<1 year n = 21	≥1 year to <2 years n = 32	≥2 years n = 5	Total n = 58
Time on therapy, median (range), months	6.2 (2.1-11.2)	23.4 (12.5-23.9)	24.7 (24.1-25.1)	14.8 (2.1-25.1)
Time to CR from treatment start date, median (range), months	2.6 (2.1-5.3)	2.8 (2.6-3.6)	2.8 (2.6-10.9)	2.8 (2.1-10.9)
Duration of CR, median (95% CI), months ^a	14.5 (8.5-16.8)	NR (31.2-NR)	13.8 (5.6-NR)	NR (16.1-NR)
Response, % ^a				
≥1 year	83.0	81.3	60.0	79.3
≥2 years	13.8	77.9	40.0	62.5
≥3 years	13.8	62.2	40.0	51.6
≥4 years	N/A	62.2	40.0	51.6

^aFrom product-limit (Kaplan-Meier) method for censored data.

Supplemental Table 4. Disposition and duration of response for patients with CR who did not receive second-course pembrolizumab.

	CR But Did Not Receive Second-Course Pembrolizumab n = 46
Disposition, n (%)	
Ongoing response	13 (28.3)
Disease progression or died	11 (23.9)
No disease assessments in 30 weeks	11 (23.9)
Started new anticancer treatment	6 (13.0)
Disease progression or died after ≥ 2 missed visits	3 (6.5)
Lost to follow-up	1 (2.2)
Received stem cell transplant	1 (2.2)
Time to response, median (range), months	2.8 (2.1-10.9)
Duration of response, median (95% CI), months ^a	NR (26.7-NR)
Response ≥ 4 years, %	67.4

^aFrom product-limit (Kaplan-Meier) method for censored data.

Supplemental Table 5. Treatment-related adverse events by cohort

n (%)	Cohort 1 n = 69	Cohort 2 n = 81	Cohort 3 n = 60	Total Population N = 210
Any-grade TRAE	54 (78.3)	52 (64.2)	47 (78.3)	153 (72.9)
TRAEs ≥5% in total population				
Hypothyroidism	6 (8.7)	12 (14.8)	12 (20.0)	30 (14.3)
Pyrexia	12 (17.4)	7 (8.6)	5 (8.3)	24 (11.4)
Fatigue	10 (14.5)	6 (7.4)	7 (11.7)	23 (11.0)
Rash	10 (14.5)	5 (6.2)	8 (13.3)	23 (11.0)
Diarrhea	9 (13.0)	4 (4.9)	4 (6.7)	17 (8.1)
Headache	10 (14.5)	3 (3.7)	3 (5.0)	16 (7.6)
Nausea	7 (10.1)	2 (2.5)	6 (10.0)	15 (7.1)
Arthralgia	4 (5.8)	5 (6.2)	4 (6.7)	13 (6.2)
Cough	3 (4.3)	4 (4.9)	6 (10.0)	13 (6.2)
Pruritus	4 (5.8)	5 (6.2)	4 (6.7)	13 (6.2)
Infusion-related reaction	6 (8.7)	3 (3.7)	2 (3.3)	11 (5.2)
Neutropenia	5 (7.2)	2 (2.5)	4 (6.7)	11 (5.2)
Grade 3 or 4 TRAEs	12 (17.4)	10 (12.3)	5 (8.3)	27 (12.9)
Grade 3 or 4 TRAEs in ≥2 patients				
Neutropenia	3 (4.3)	2 (2.5)	0	5 (2.4)
Pericarditis	2 (2.9)	0	0	2 (1.0)

Diarrhea	1 (1.4)	1 (1.2)	0	2 (1.0)
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TRAE, treatment-related adverse event.

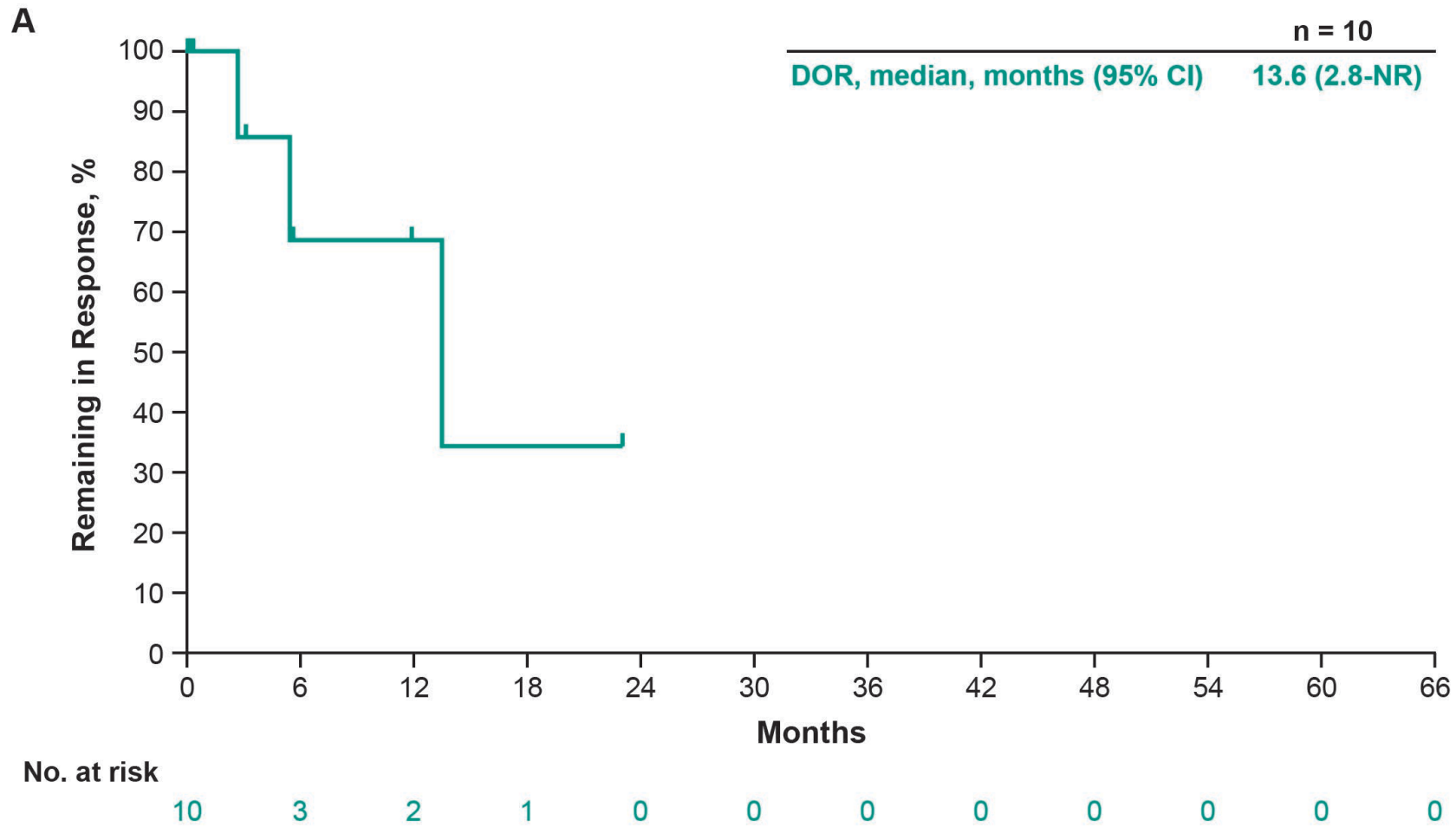
Supplemental Table 6: Treatment-related adverse events for patients who received second-course pembrolizumab

n (%)	Received Second-Course Pembrolizumab n = 20
Any-grade TRAE	19 (95.0)
TRAEs in ≥ 2 patients	
Fatigue	4 (20.0)
Diarrhea	3 (15.0)
Muscle spasms	3 (15.0)
Rash	3 (15.0)
Arthralgia	2 (10.0)
AST increased	2 (10.0)
Constipation	2 (10.0)
Hypomagnesaemia	2 (10.0)
Hypophosphatemia	2 (10.0)
Pericarditis	2 (10.0)
Platelet count decreased	2 (10.0)
Pruritus	2 (10.0)
Upper respiratory tract infection	2 (10.0)
Grade 3 TRAEs	6 (30.0)
Grade 3 TRAEs in ≥ 1 patient	
Pericarditis	2 (10.0)

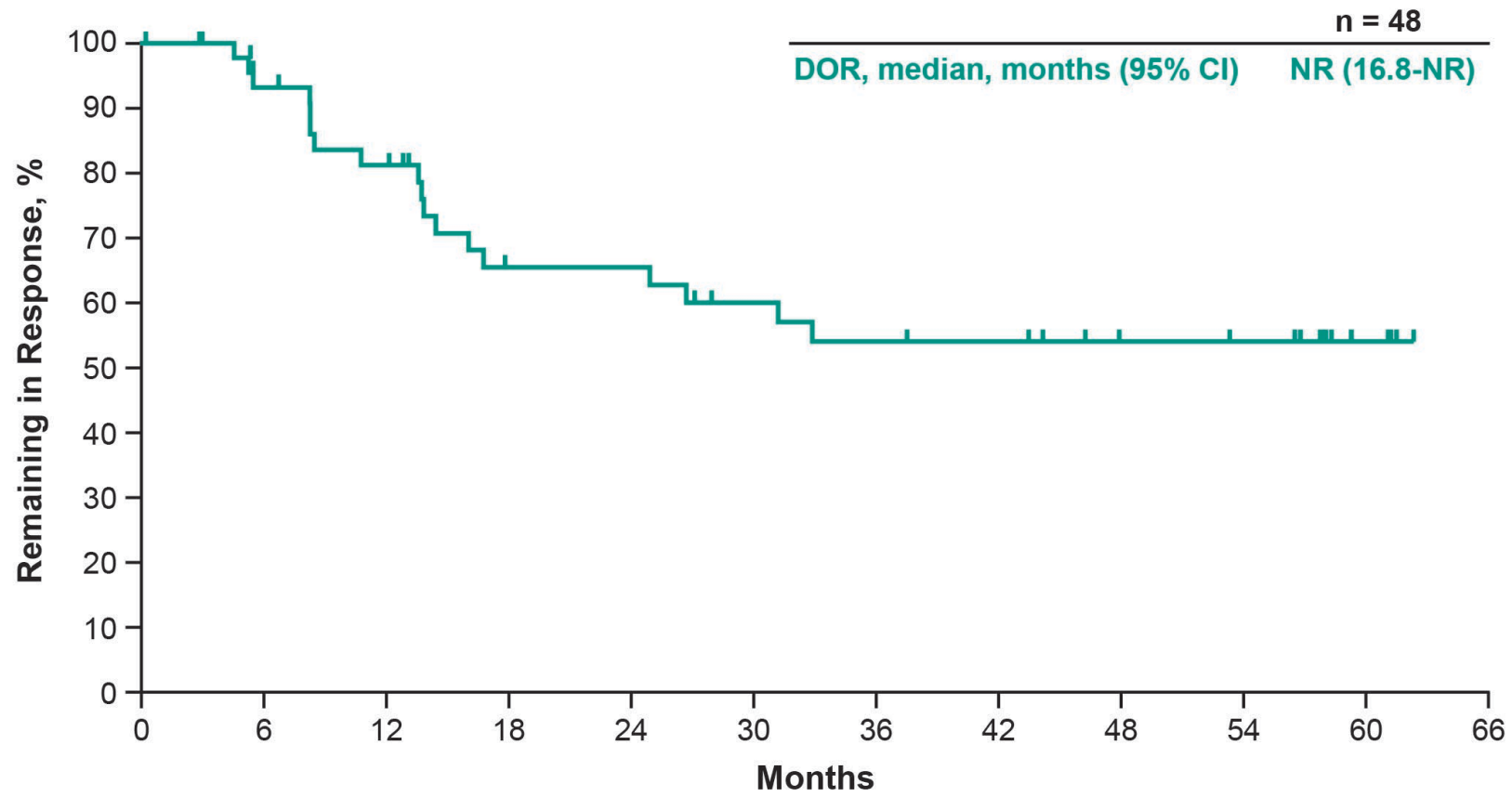
Colitis	1 (5.0)
Diarrhea	1 (5.0)
Gastrointestinal pain	1 (5.0)
Neutropenia	1 (5.0)
Peripheral neuropathy	1 (5.0)

TRAE, treatment-related adverse event.

Supplemental Figure 1. Kaplan-Meier estimates of duration of response (DOR) in patients who achieve complete response (CR) and (A) received allogeneic stem cell transplant (B) did not receive allogeneic stem cell transplant



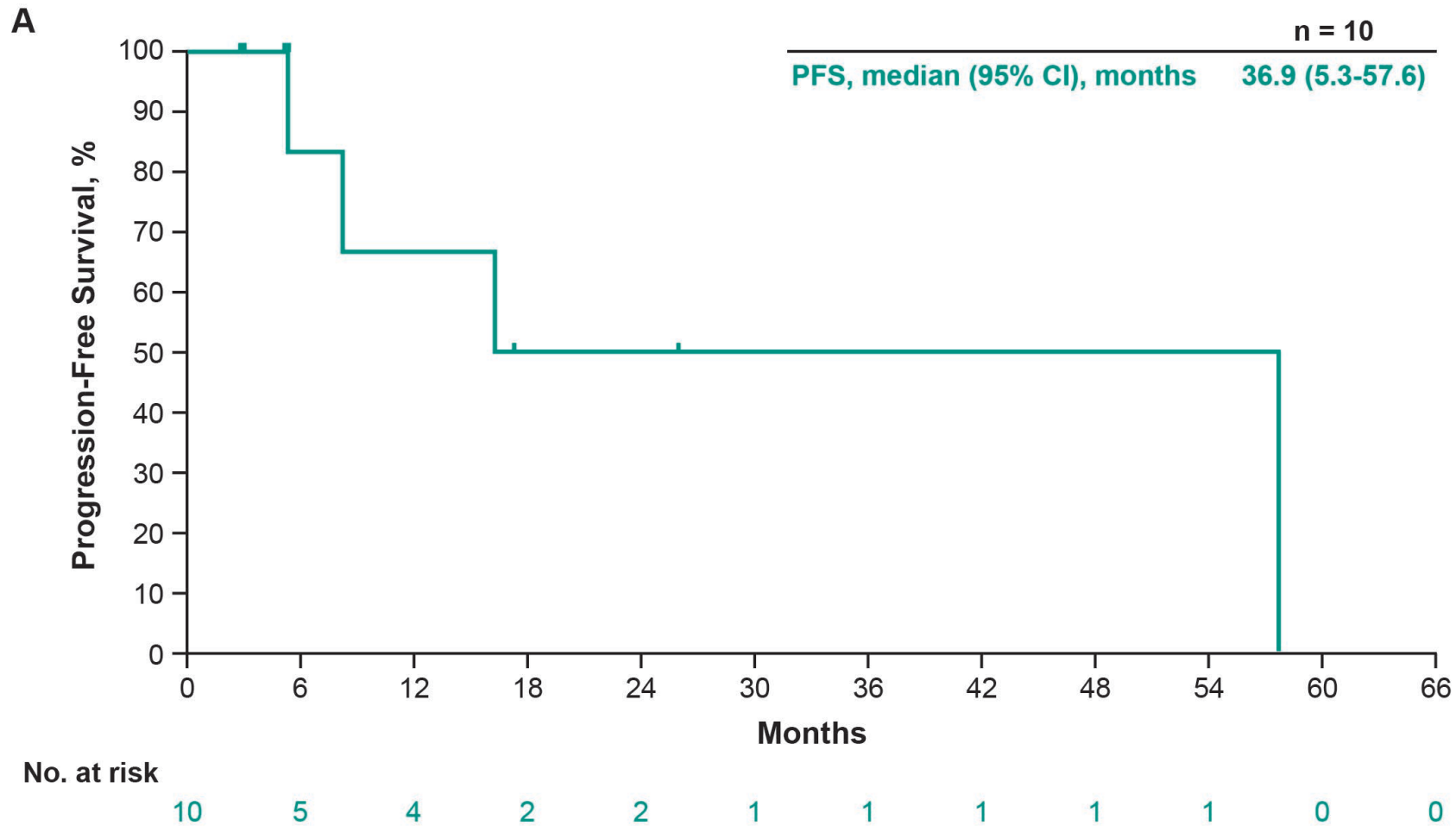
B



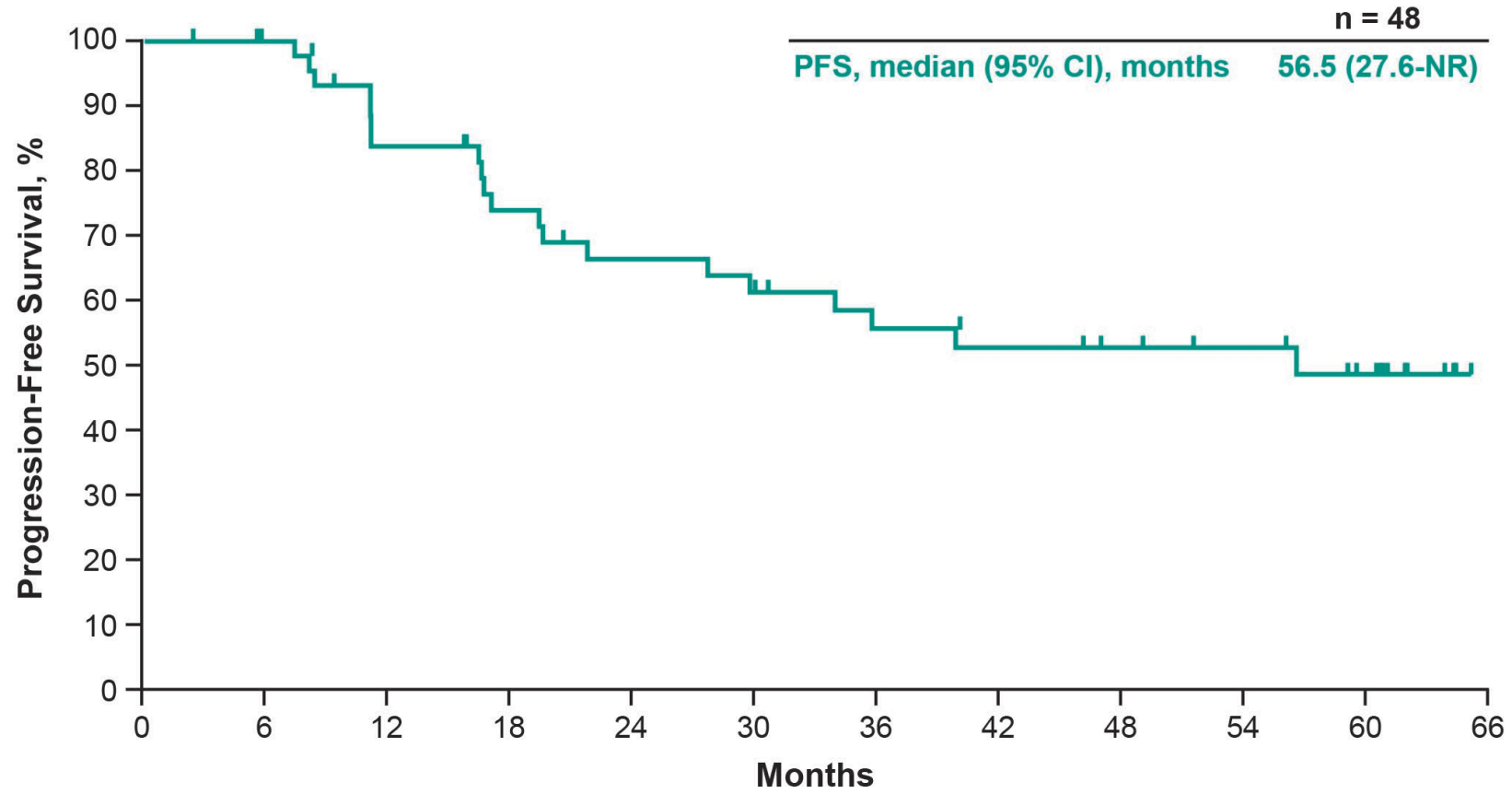
No. at risk

48 40 33 24 24 20 18 17 13 12 4 0

Supplemental Figure 2. Kaplan-Meier estimates of progression-free survival (PFS) in patients who achieved complete response (CR) and (A) received allogeneic stem cell transplant (B) did not receive allogeneic stem cell transplant.



B



No. at risk

48 46 36 30 26 23 20 18 16 14 10 0

Supplemental Figure 3. Time to response and response duration for patients who received a second course of pembrolizumab. CR,
 complete response; PR, partial response; SD, stable disease; PD, progressive disease

