

# Hospitalization and healthcare resource use of omidubicel vs cord blood transplantation for hematologic malignancies in a global randomized phase III clinical trial

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## INTRODUCTION

- Umbilical cord blood (UCB) remains an important source of hematopoietic stem cells for patients where no matched donor is available and for racial minorities. However, cord blood transplants have been associated with delayed hematopoietic recovery, prolonged hospitalization, and higher costs of transplant compared with other donor sources
- Omidubicel is an advanced cell therapy that preserves stem cell function to optimize cell homing, engraftment, differentiation, and self-renewal and is manufactured from an appropriately matched human leukocyte antigen-matched single UCB unit for each patient
- A recent phase III clinical trial (NCT02730299) reported that patients who received omidubicel experienced faster time to neutrophil engraftment, faster platelet recovery, reduced rates of infections, and shorter hospitalization time than patients who received standard single (33%) or double (67%) UCB transplantation<sup>1</sup>
- We report on an analysis of resource utilization including hospital length of stay, hospital care setting, visits by provider type, rates of transfusions, and readmissions from the phase III trial

## METHODS

- The phase III clinical trial included patients 12–65 years of age with hematologic malignancies and eligible for allogeneic transplant. The primary endpoint was time to neutrophil engraftment, with secondary endpoints of time to platelet engraftment, incidence of first bacterial infection (grades 2–3) or invasive fungal infection, and days alive and out of hospital in the first 100 days. 125 patients were randomized in the study
- We analyzed resource utilization data for patients treated with omidubicel (n=52) or UCB (n=56) (“as-treated” population), focusing on resource utilization within the first 100 days of transplantation
- Summary statistics were compared between treatment arms, means and medians were used to draw comparisons and significance testing was performed

## RESULTS

- The demographics and patient characteristics were well-balanced overall, with slightly more males and a lower median age in the UCB arm (Table 1). The study population was diverse, with over 40% non-White participants
- In the phase III clinical trial, omidubicel met its primary endpoint and secondary endpoints with statistical significance (Table 2).<sup>1</sup> The rates of acute and chronic graft-versus-host disease in the 2 arms were comparable
- Within the first 100 days after transplant, patients receiving omidubicel experienced shorter average total length of hospital stay than UCB recipients (mean, 41.2 vs 50.8 days;  $P=0.027$ ) and more days alive and out of the hospital (mean, 55.8 vs 43.7 days;  $P=0.023$ ) (Figure 1; Table 3). Twelve percent of patients in the omidubicel arm died vs 16% in the UCB arm before day 100
- During the primary hospitalization (transplant to discharge), fewer patients receiving omidubicel required intensive care unit (ICU) stay (9.6% vs 23.2%) compared with UCB recipients (Figure 2)
- Patients receiving omidubicel required fewer outpatient consultant visits and fewer outpatient non-consultant visits (eg, X-rays, scans, biopsies) (Table 4) and required fewer platelet or other transfusions (red blood cell, albumin, plasma, and factor product) within 100 days from transplant (Figure 3)

**TABLE 1. CHARACTERISTICS OF PATIENTS TREATED WITH Omidubicel OR UCB IN THE PHASE III CLINICAL TRIAL**

Characteristic	Omidubicel		UCB	
	n	%	n	%
<b>Total treated</b>	<b>52</b>	<b>100</b>	<b>56</b>	<b>100</b>
Male	25	48.1	35	62.5
<b>Age, years</b>				
Median, years (range)	40 (13–62)		36 (13–64)	
12–17	7	13.5	7	12.5
18–39	19	36.5	23	41.1
40–65	26	50.0	26	46.4
<b>Race/Ethnicity</b>				
White	31	59.6	29	51.8
Black	10	19.2	9	16.1
Asian	5	9.6	11	19.6
Hispanic or Latino	9	17.3	6	10.7
Other/Unknown	6	11.5	7	12.5
<b>HCT-specific comorbidity index</b>				
0	10	19.2	13	23.2
1–2	15	28.8	15	26.8
3+	27	51.9	28	50.0
<b>Karnofsky/Lansky performance score</b>				
90 or above	40	76.9	42	75.0
<90	12	23.1	14	25.0
<b>Primary diagnosis</b>				
AML	22	42.3	27	48.2
ALL	18	34.6	19	33.9
CML	4	7.7	2	3.6
MDS	5	9.6	3	5.4
Lymphoma	2	3.8	3	5.4
Hodgkin lymphoma	0	0	1	1.8
Non-Hodgkin lymphoma	2	3.8	2	3.6
Other rare disease	1	1.9	2	3.6

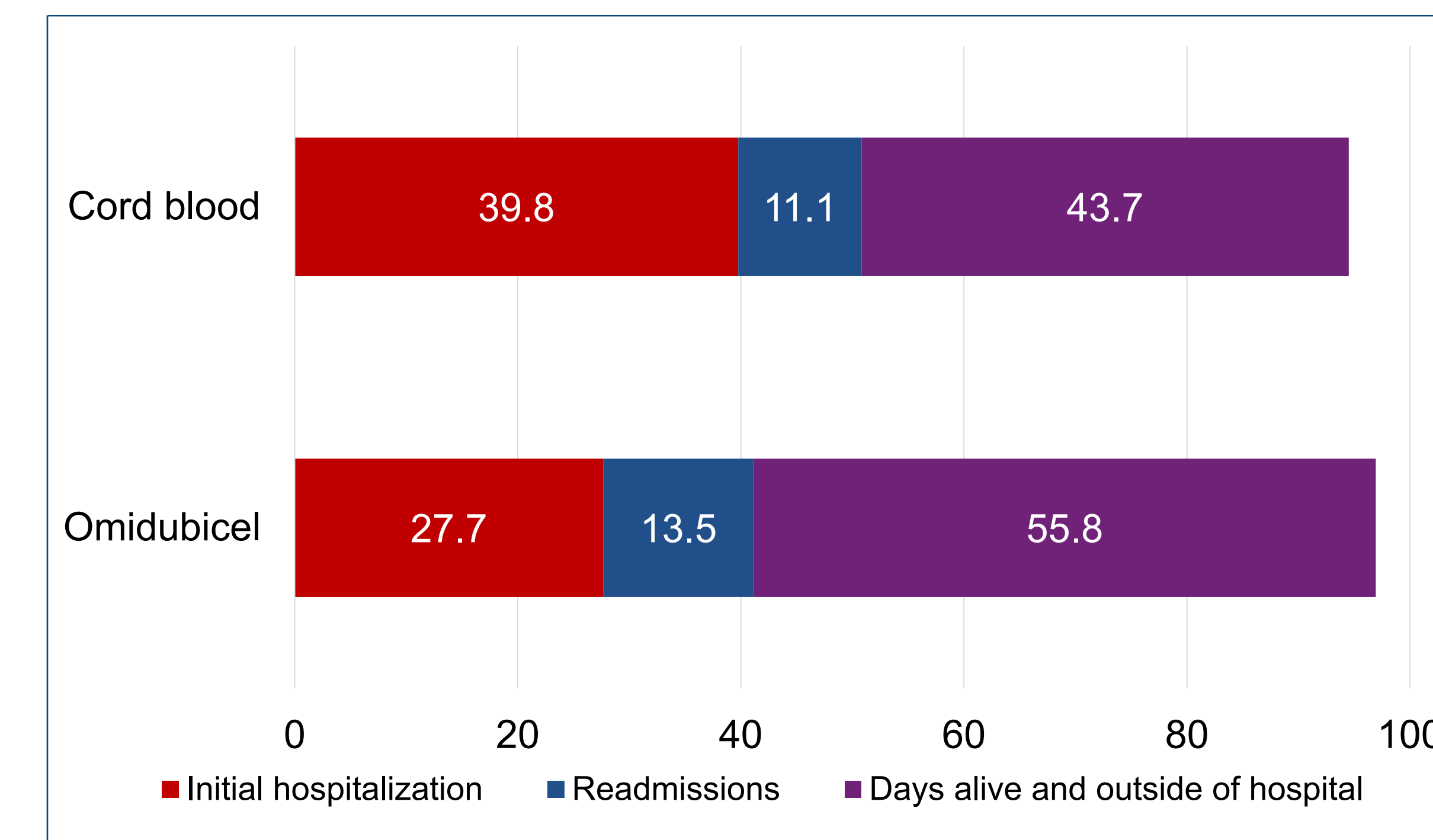
ALL: acute lymphoblastic leukemia; AML: acute myelogenous leukemia; CML: chronic myelogenous leukemia; HCT: hematopoietic cell transplantation; MDS: myelodysplastic syndromes; UCB: umbilical cord blood.

**TABLE 2. PRIMARY AND SECONDARY ENDPOINTS FROM A PHASE III CLINICAL TRIAL OF Omidubicel VS UCB TRANSPLANTATION IN PATIENTS WITH HEMATOLOGIC MALIGNANCIES IN THE ITT POPULATION<sup>1</sup>**

	Omidubicel (n=62)	UCB (n=63)	P value
Median time to neutrophil engraftment, days (95% CI)*	12 (10.0–14.0)	22 (19.0–25.0)	<0.001
Cumulative incidence of platelet engraftment by day 42, %	55	35	0.028
Cumulative incidence of first grade 2–3 bacterial or invasive fungal infections during the first year after transplant, %	37	57	0.03
Median time outside the hospital during the first 100 days post-transplant, days	61	48	0.005
Cumulative incidence of first grade 3 viral infection during the first year after transplant, %	10	26	0.02

\*Patients not transplanted or who did not engraft on/before day 42 after transplantation were assigned to day 43. CI: confidence interval; ITT: intent-to-treat; UCB: umbilical cord blood.

**FIGURE 1. DAYS IN AND OUT OF HOSPITAL FROM DAY 0 TO DAY 100**

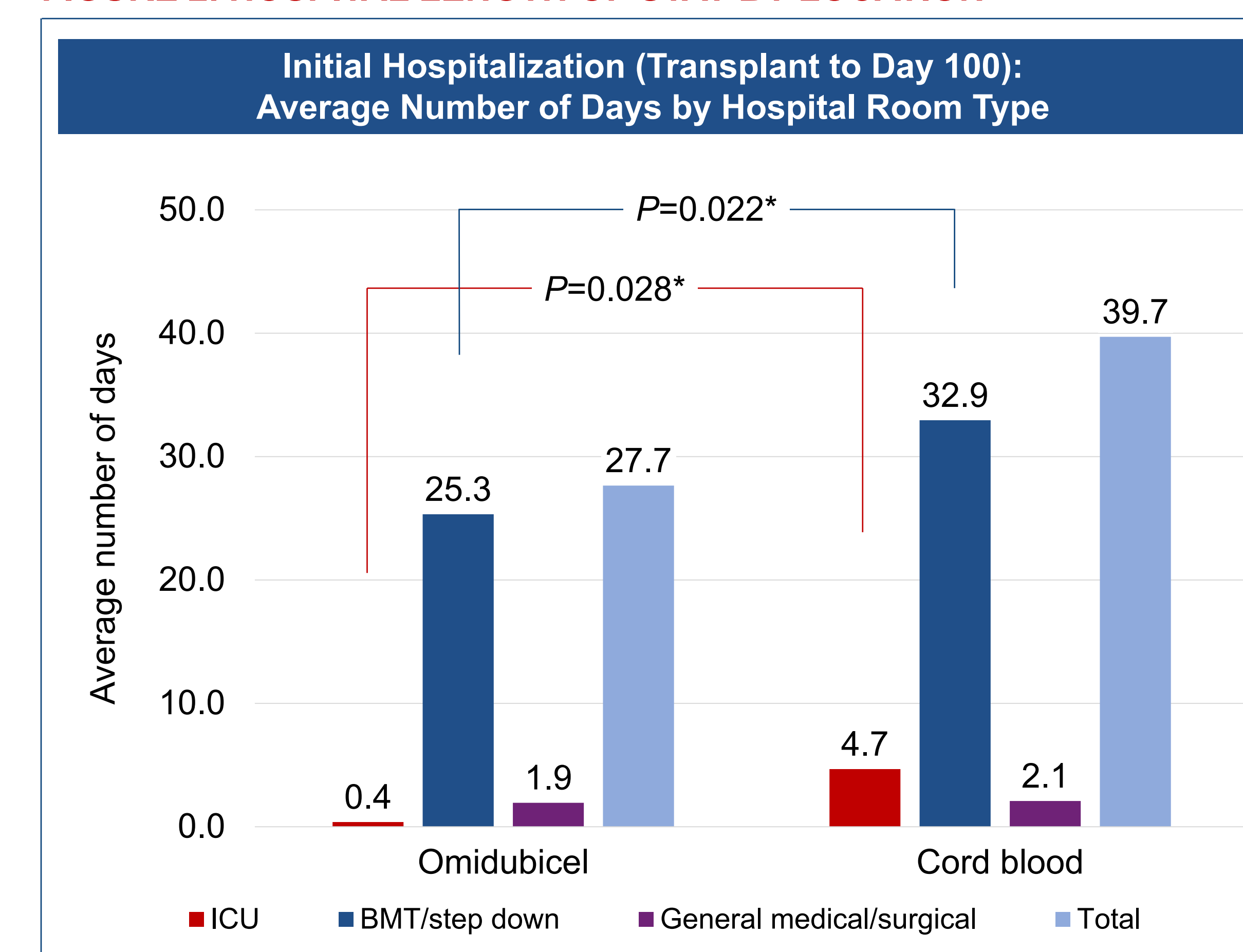


**TABLE 3. RESULTS DAYS 0–100**

	Omidubicel (n=52)	UCB (n=56)	P value
Median time to neutrophil engraftment, days	10	20.5	<0.001*
Acute GVHD in first 100 days	15%	20%	0.563*
Mean total number of inpatient days during primary hospitalization (transplant to discharge) within first 100 days post-transplant	27.7	39.8	<0.001†
Mean total number of inpatient days within first 100 days post-transplant (includes readmissions)	41.2	50.8	0.027‡
Mean total days alive and not hospitalized within 100 days post-transplant	55.8	43.7	0.023‡

\*Per protocol population. †Significant at 99% confidence. ‡Significant at 95% confidence. GVHD: graft-versus-host disease; UCB: umbilical cord blood.

**FIGURE 2. HOSPITAL LENGTH OF STAY BY LOCATION**



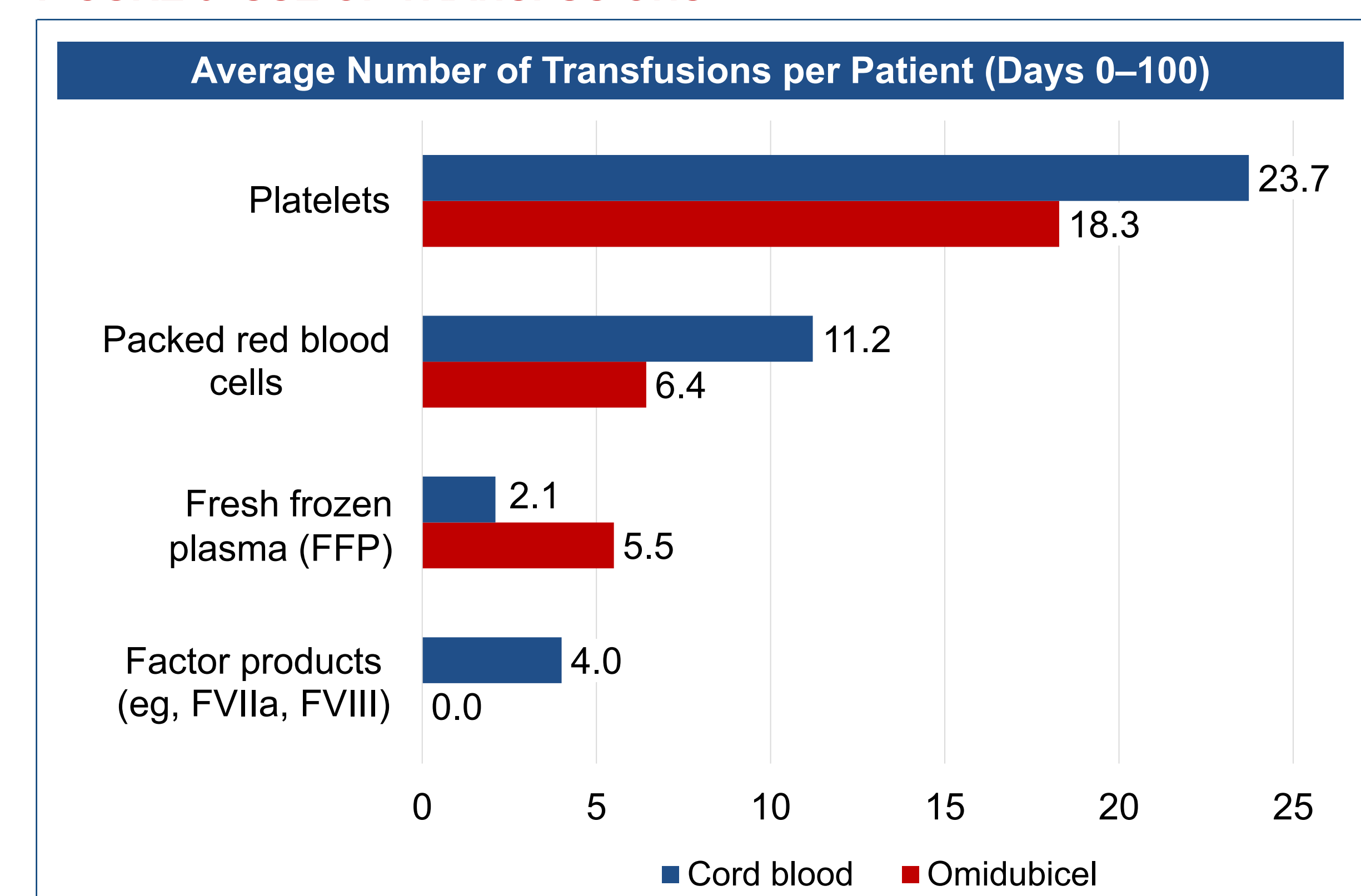
\*Significant at 95% confidence interval. BMT: bone marrow transplant; ICU: intensive care unit.

**TABLE 4. OUTPATIENT CLINICAL VISITS AND PROCEDURES**

	Omidubicel (n=52)	UCB (n=56)	P value
Average number of consultant visits <sup>a</sup>	6.8	20.1	0.015*
Average number of non-consultant/outpatient visit procedures <sup>b</sup>	15.3	24.0	0.025*
<b>Transfusions</b>			
Average number of platelet transfusions per patient	18.3	23.7	0.051
Average number of transfusions per patient (any type)	24.8	35.4	0.005†

\*Significant at 95% confidence. †Significant at 99% confidence. <sup>a</sup>Including the following: ID, dermatology, GI, cardiology, neurology, and surgery consultant visits. <sup>b</sup>Outpatient clinic visits or outpatient procedures (ie, X-rays, ultrasounds, CT scans, PET scans, MRIs, MRAs, EGDS, colonoscopy or any lower GI scope, ventilations, dialysis, biopsies, bone marrow exams, cytogenetics, molecular markers). CT: computed tomography; EGD: esophagogastroduodenoscopy; GI: gastrointestinal; MRA: magnetic resonance angiogram; MRI: magnetic resonance imaging; PET: positron emission tomography; UCB: umbilical cord blood.

**FIGURE 3. USE OF TRANSFUSIONS**



## CONCLUSION

- This analysis shows that more rapid neutrophil engraftment in patients transplanted with omidubicel was associated with significantly shorter hospital length of stay, reduced stays in ICU settings, and reduced healthcare resource use compared with UCB in the clinical trial
- Although economic data were not collected as part of the clinical trial, the costs of providing transplantation care during the first 100 days are likely lower with omidubicel compared with UCB in the real-world setting, as hospital stay, outpatient visits, and blood product transfusions are among the major drivers of costs during this period

## REFERENCE

1. Horwitz ME, et al. *Blood*. 2021;138:1429–1440.

