



OSSIUM HEALTH[®]

Transfusion of Cryopreserved Organ Donor-derived Bone Marrow

ICD-10 Coordination & Maintenance Committee | Fall 2026

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CEO, Co-Founder & President

Bone marrow transplants are a well-established curative treatment, but significant unmet needs remain

Allogeneic hematopoietic cell transplantation (AlloHCT) remains the only potentially curative therapy for various high-risk hematologic malignancies, aplastic anemia, and sickle cell disease

GRAFT FAILURE

Between 5% and 12% of patients experience primary or secondary graft failure.¹

GRAFT VS HOST DISEASE

Patients experienced acute GVHD in 31% of cases and moderate-to-severe chronic GVHD in 27%.²

DONOR AVAILABILITY

41% of patients do not find a matching donor, and 9.4% of matched donors drop out.⁴

RELAPSE RATES

Relapse rates of up to 30% are observed within 1 year post-transplant.²

INFECTION

Pre-engraftment infections occurred in 47.9% of patients, due to immune system ablation.³

TRANSPLANT DELAYS

Transplant delays can cause a 20% drop in 3-year survival (70%→50%) and raise progression risk.⁵

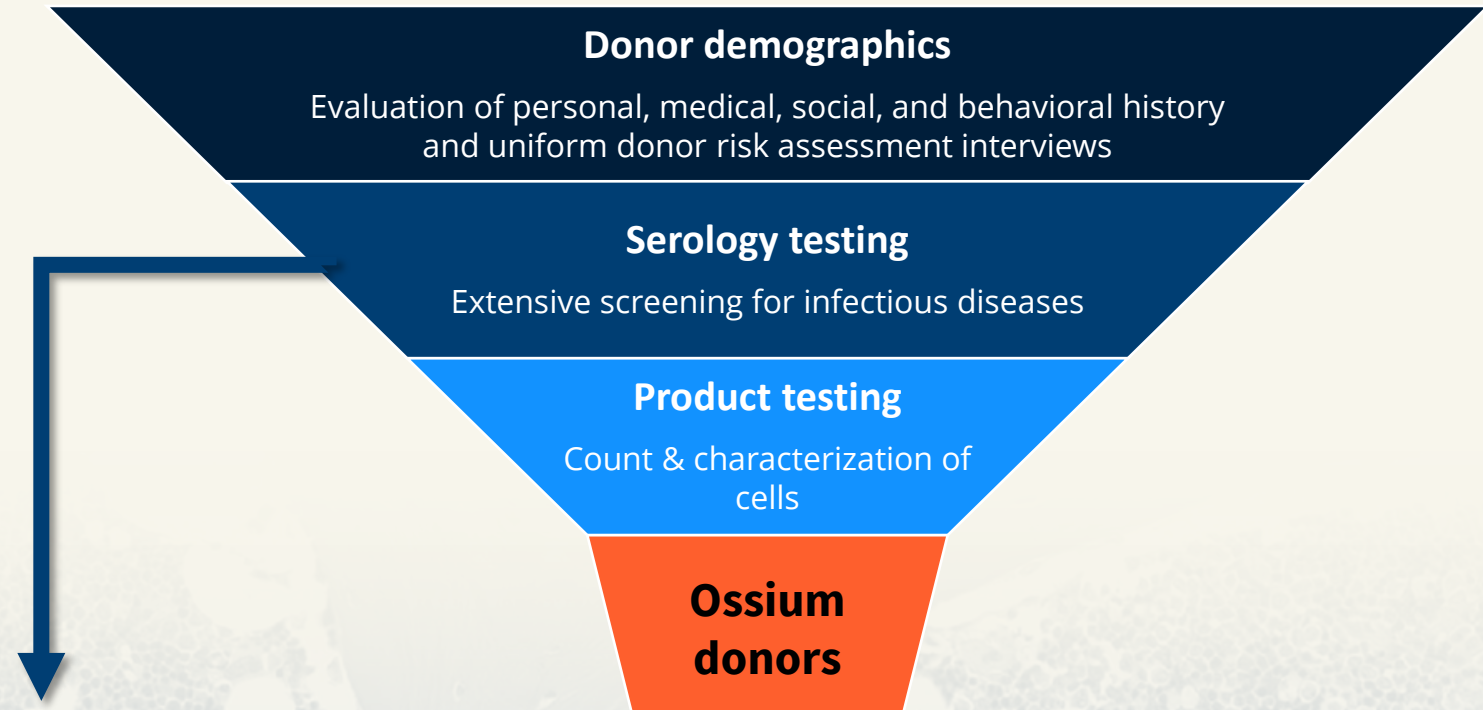
¹Novitzky-Basso I, Al-Shaibani E, Remberger M et al. Risk factors for graft failure in allogeneic hematopoietic stem cell transplantation: a single-center study. *Cell Ther Transplant*. 2020;9(4):37–47.

²Shaw BE, Jimenez-Jimenez AM, Burns LJ, et al. National Marrow Donor Program-Sponsored Multicenter, Phase II Trial of HLA-Mismatched Unrelated Donor Bone Marrow Transplantation Using Post-Transplant Cyclophosphamide. *J Clin Oncol*. Published online April 27, 2021. doi:10.1200/JCO.20.03502 ³Young JAH, Logan BR, Wu J, Wingard JR, Weisdorf DJ, Mudrick C, et al. Infections after transplantation of bone marrow or peripheral blood stem cells from unrelated donors. *Biol Blood Marrow Transplant*. 2016;22(3):359–370. ⁴Balassa, K. & Griffiths, A. & Winstone, D. & Li, Y. & Rocha, Vanderson & Pawson, R.. (2019). Attrition at the final donor stage among unrelated haematopoietic stem cell donors: the British Bone Marrow Registry experience. *Transfusion Medicine*. 29. 10.1111/tme.12613. ⁵Geng L, Chen E, Ji Y, Liu H, Sun Z. Optimizing Timing and Preparation for Allogeneic Hematopoietic Stem Cell Transplantation in Higher-Risk Myelodysplastic Syndromes. *Blood Lymphat Cancer*. 2025;15:103-113.

Ossium leverages the **robust organ donation ecosystem** to access **high-quality donors**

Donor Inclusion Criteria

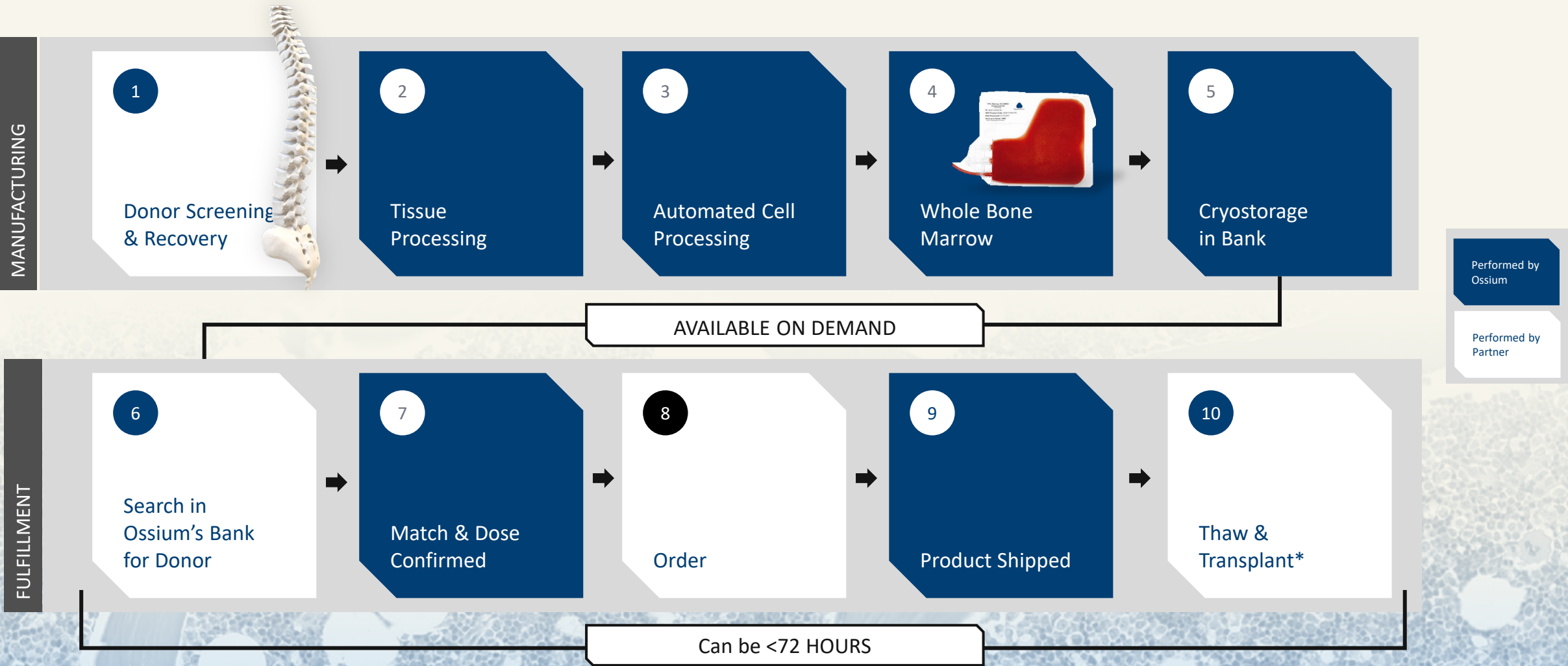
1. Those who donated after brain death (DBD) or after circulatory death (DCD).
2. Male and female donors aged **7-55 years old** who have authorization or authorized for organ and tissue procurement.
3. Maximum warm ischemia time: **8 hours**.
4. Maximum cold ischemia time: **84 hours**.
5. Donors undergo **eligibility screening procedures**, including an evaluation of medical history and relevant social behavior, per 21 CFR 1271 and the FDA's Guidance for Industry.



Serology testing includes:

- Hepatitis B Surface Antigen (HBsAg)
- Hepatitis B Core Antibody (HBc Ab)
- Human T-Lymphocyte Virus Type I and II (HTLV I/II)
- Human Immunodeficiency Virus 1 and 2 Plus O Antibody
- Hepatitis C Virus Antibody (Anti-HCV)
- Syphilis or Serological Test for Syphilis (STS)
- HIV 1 / HCV / HBV Nucleic Acid Testing (NAT)
- West Nile Virus (WNV) NAT
- Chagas – Anti-T. cruzi Assay
- Cytomegalovirus (Anti-CMV, CMV IgM)
- Epstein-Barr Virus (EBV VCA IgG or EBNA)
- Toxoplasmosis (IgG)

Ossium's Organ Donor-Derived HPC, Marrow is banked in advance, enabling <72-hour search to transplant



*Documented in the medical record and healthcare professional notes in the same manner as for other HPC allografts.

As the FDA does not regulate bone marrow transplants from living donors per CFR 1271.3(d)(4), Organ Donor HPC, Marrow does not require a Biologics License Application (BLA)

The Center for Biological Evaluation and Research (CBER) Tissue Reference Group's (11 August 2017) response to Ossium's request for FDA guidance (potential regulatory pathway and classification):

*"21 CFR 1271.3(d)(4) states that minimally manipulated bone marrow for homologous use and not combined with another article (except for water, crystalloids, or a sterilizing, preserving, or a storage agent, if the addition of the agent does not raise new clinical safety outcomes with respect to the bone marrow) is not considered to be a human cell, tissue, or cellular or tissue-based product (HCT/P). **[HPC, Marrow], when indicated for hematopoietic and immunologic reconstitution (i.e., for homologous use), is not considered an HCT/P and does not require FDA oversight**"*

CBER confirmed classification upon Ossium's re-engagement following full development of HPC, Marrow (January 2020):

*"Based on the additional information provided..., the **HPC, Marrow** (Cryopreserved) product described, **when indicated for hematopoietic and immunologic reconstitution (i.e., for homologous use) does not appear to be an HCT/P**"*



Organ Donor HPC, Marrow clinical program

PRESERVE I

- Pre-market, first-in-human study to demonstrate safety and feasibility of cryopreserved organ donor-derived bone marrow transplantation
- Cohort 1 (N=4); Cohort 2 (N=8): RIC or MAC, 36 months follow-up

FULLY ENROLLED

HOPE Expanded Access Program

(HPC Offered for PRESERVE Expansion)

- For patients unable to join the PRESERVE I study due to eligibility or logistical issues or sites not participating in PRESERVE I
- Day 180 post-transplant data presented at Tandem 2026

IN PROGRESS

PRESERVE II

- Larger study to measure long-term efficacy outcomes compared to living donor recipients (25 sites)
- Patients will be enrolled in a ratio of 1:2 (Organ Donor HPC, Marrow: Living Donor)

CURRENTLY PLANNING

Publications and conference presentations:

Cytotherapy 28 (2026) 102006: First 3 Organ Donor HPC, Marrow patients (HOPE expanded access program);
Other data presented at Tandem 2026, ASH 2025, EHA 2025, AOPO 2025

PRESERVE I:

A First-in-Human Study of HLA-Partially to Fully Matched Allogenic Cryopreserved Organ Donor-derived Bone Marrow Transplantation for Patients with Hematologic Malignancies

Enrollment Phase



- Adults aged **18 - 75**
- **HLA partially or fully matched (4-8/8)**
- Diagnosed with **ALL, AML, ABL, AUL, MDS without fibrosis, CML, and CLL, chemosensitive lymphoma with adequate organ function**
- HCT-CL ≤ 5



- Availability of **suitable graft** from living donor
- Prior autologous or allogeneic HCT
- Presence of DSA's
- Uncontrolled Infection

Main Phase: Treatment Period with HPC, Marrow

1

Conditioning

Pre-HCT: myeloablative conditioning or reduced intensity conditioning per site's SOP

2

Transplantation Dose

Cell dose is 2.5 to 6×10^6 CD34+ per kg patient weight

3

Follow Up Post-HCT

GVHD prophylaxis consisting of:

- Cyclophosphamide 50mg/kg IV on Day 3 & 4
- Tacrolimus 0.03mg/kg IV on Day 5 (adjusted for IBW)
- Mycophenolate Mofetil (MMF) 15mg/kg PO TID on Day 5

Close-Out Phase

Primary Endpoints

- Neutrophil engraftment by Day 28
- CTCAE Grade 3 or higher adverse events (AEs) by Day 28 (related to infusion of product)
- CTCAE Grade 3/4 AEs by Day 56 attributed to Ossium product
- Serious adverse events (SAEs)
- Death

Secondary Endpoints

- Platelet recovery by Day 56
- 1-year disease relapse and survival rate
- Transplant-related mortality
- aGVHD and cGVHD
- Clinically significant infections

Study Stopping Rules

- If two patients experience a SAE related to the IP
- One death attributed to the IP

Summary Patient Demographics and Interim Results

Parameters	Results
Total n	25
Disease Status, n (%)	
AML	13 (52)
MDS	5 (20)
Other (B-cell ALL, T-cell ALL, HTLV ATLL,DLBCL)	6 (24)
Non-Malignant Condition	1 (4)
Age Range (years)	14-69
Gender, n (%)	
Female	10 (40)
Male	15 (60)
Race, n (%)	
White	9 (36)
Black or African American	12 (48)
Asian	2 (8)
Unknown	2 (8)
Conditioning Regimen, n (%)	
Myeloablative Conditioning (MAC)	9 (36)
Reduced Intensity Conditioning (RIC)/NMA	16 (64)
HLA Match Degree, n (%)	
4/8	21 (84)
5/8	3 (12)
6/8	1 (4)

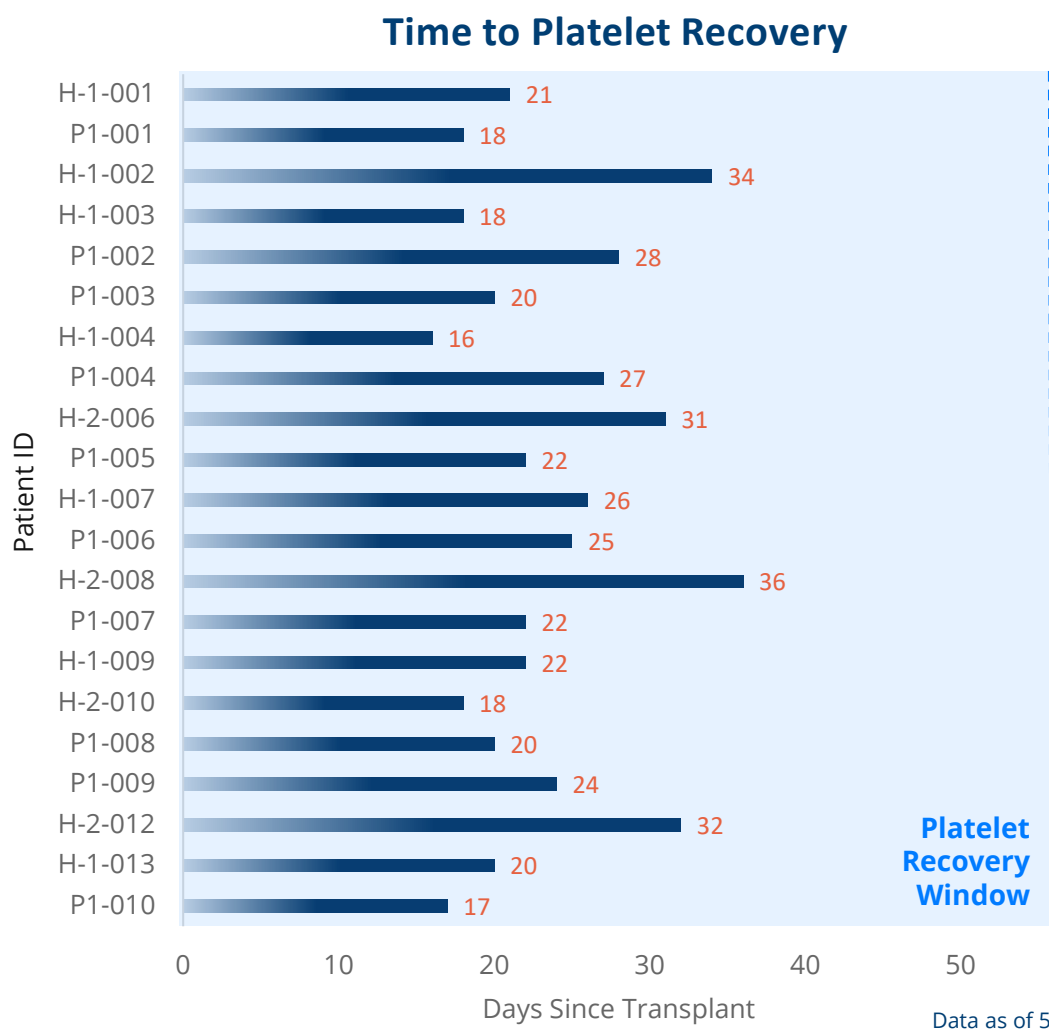
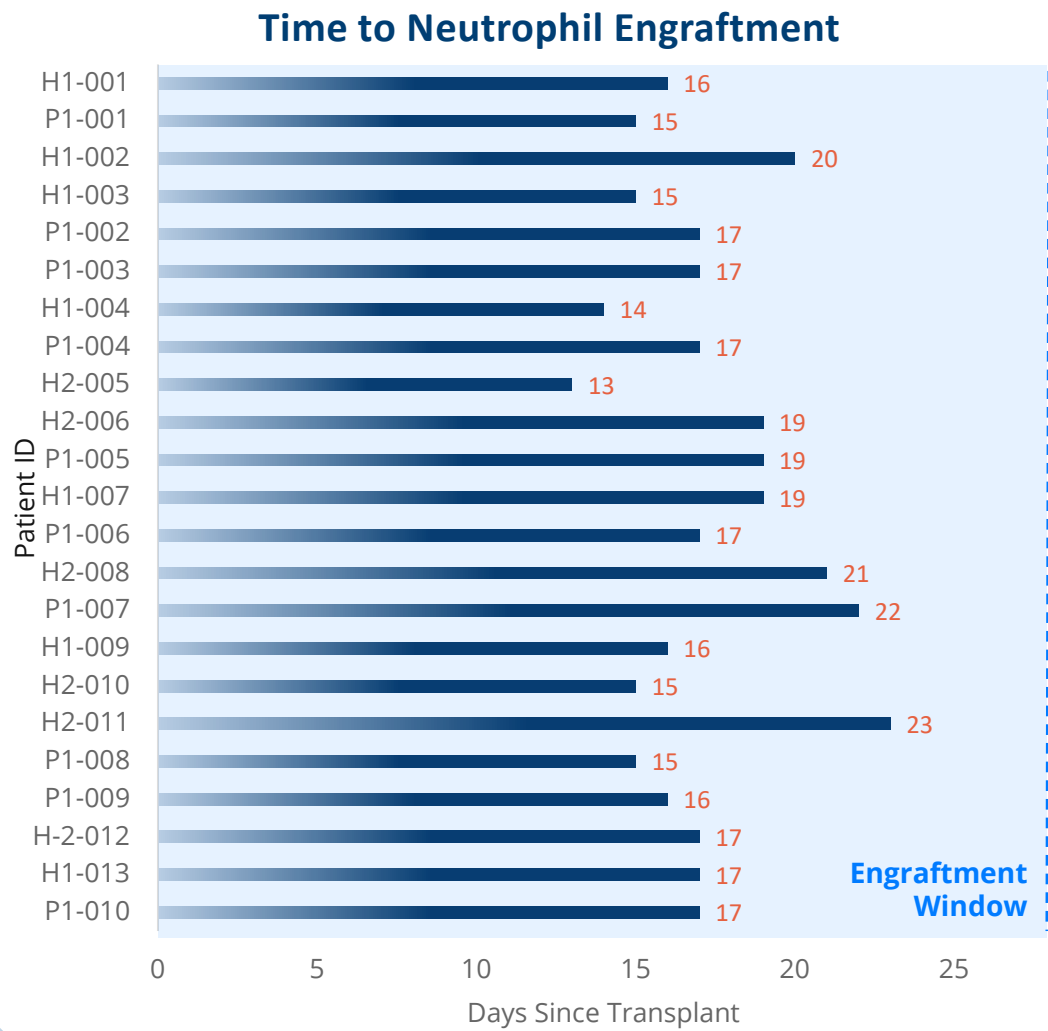
Outcome	PRESERVE-I Trial	HOPE Expanded Access
Patients Enrolled (n)	10	15
Neutrophil Engraftment (%)	100	100
Completed six months follow up (n)	4	4
Completed one year follow up (n)	2	2
Relapse by Day 365 (n)	0	1*
Death reported within one year (n)	0	2** (bacterial infection)
Primary graft failure (n)	0	0
Secondary graft failure (n)	1 (viral reactivation)	0
Number of patients meeting stopping rules (n)	0	N/A
Grade III aGVHD (n)	1	2
Grade IV aGVHD (n)	0	1 (patient received 25mg/kg PTCy)
cGVHD (n) requiring systemic steroids	1 (mild)	1 (mild)
Infection (CTCAE Grade 3 or higher for CMV, Bk cystitis, EBV, or Adenovirus)	2	Pending
Infusion Reactions	0	0

* Patient had active disease (3% circulating blasts) when receiving transplant.

** Not related to Ossium's Organ Donor HPC, Marrow.

Data as of 5/4/2026. Trial in Progress

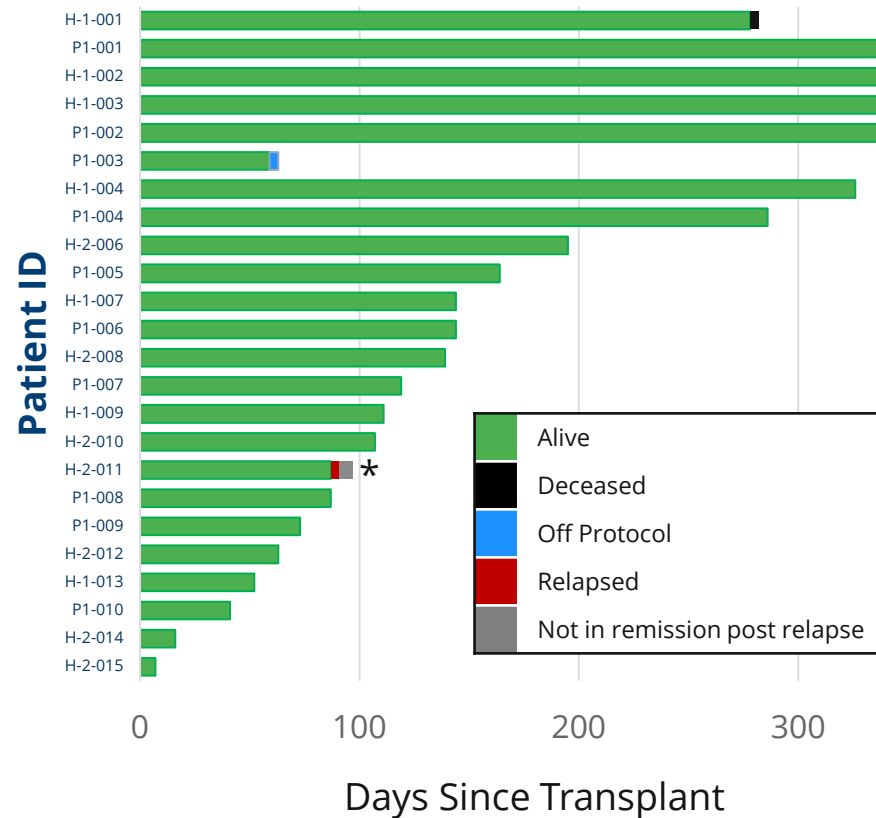
All patients to date have achieved neutrophil engraftment and platelet recovery within the specified windows



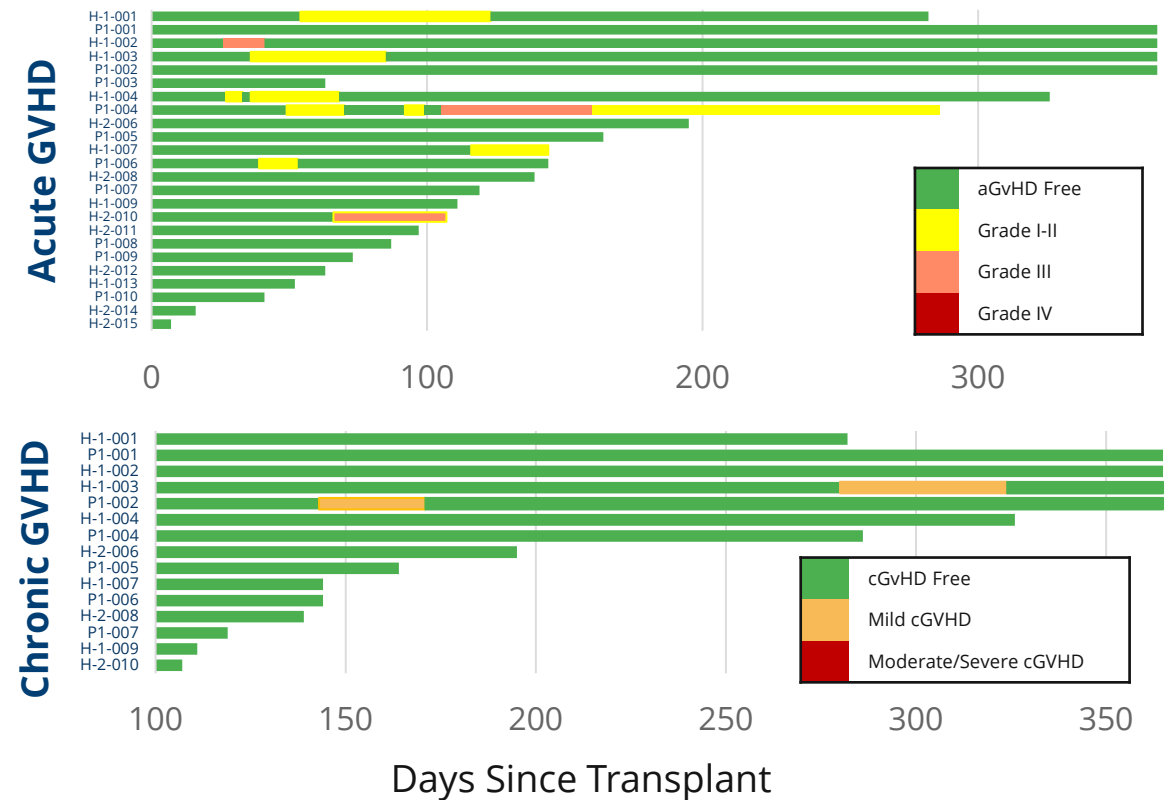
Patient H-2-005 was given 50% of standard protocol defined dose (25 mg/kg IV) PTCy due to cardiac risk and is excluded from this analysis.

No safety events related to Ossium's product have been reported; GVHD has been manageable to date

Survival and Relapse Status



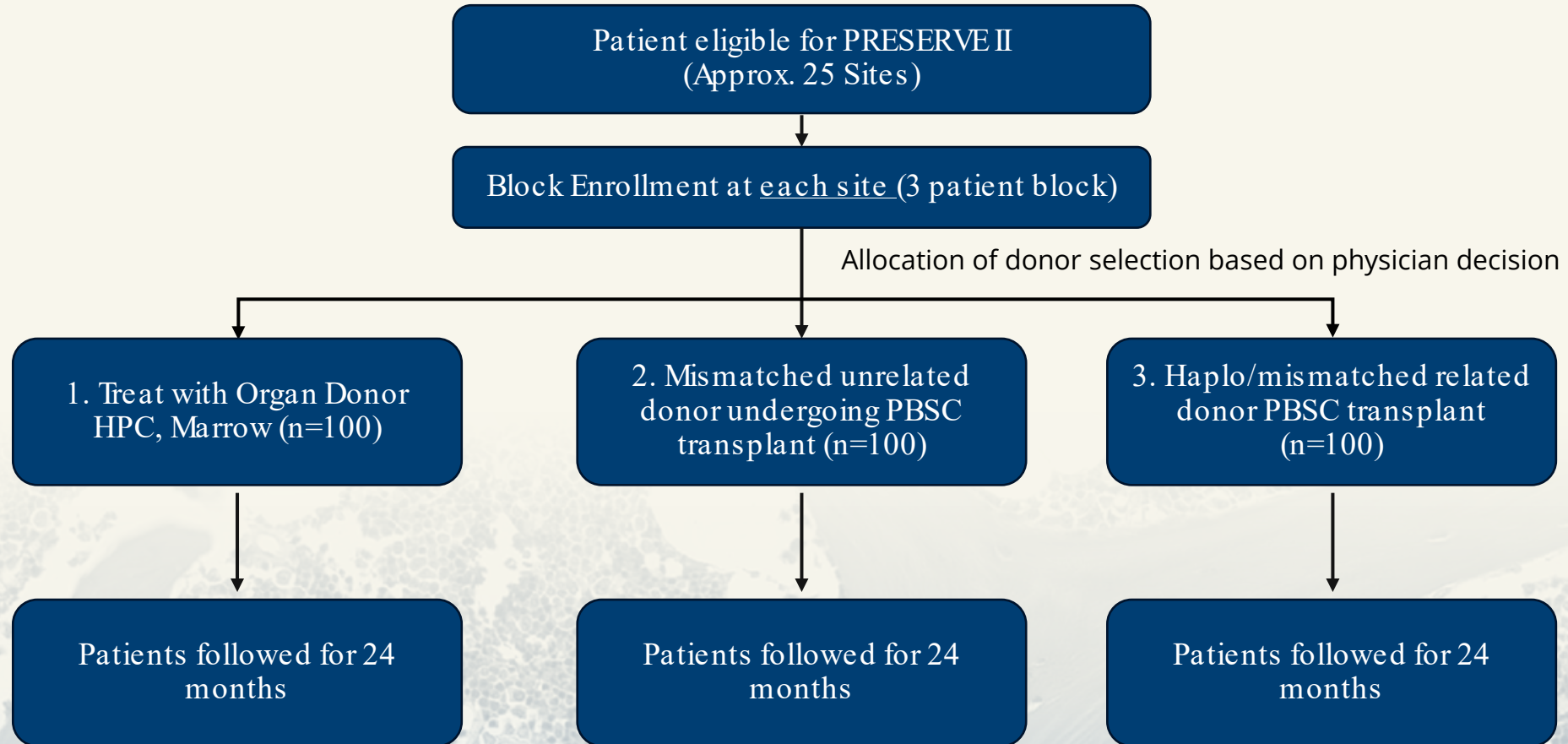
GVHD Status



Data as of 5/4/2026

*Patient H-2-011 had active disease (3% circulating blasts).
Patient H-2-005 was given 50% of standard protocol defined dose (25 mg/kg IV) PTCy due to cardiac risk and is excluded from this analysis.

PRESERVE II – Patient Workflow



New block(s) will be opened if :

1. If patient is eligible to be treated with Organ Donor HPC, Marrow or
2. If a block of 4 patients as noted above is completed

Off-the-shelf, cryopreserved Organ Donor HPC, Marrow can address patients with these unmet needs

1

Chemo Cycle Limitations

When additional chemo cycles are not preferred or tolerated

2

Donor Falling Through

After patient is conditioned

3

High-Risk MDS/AML

Narrow treatment window

4

Partial Remission

Eligible for allogeneic transplant

5

Refractory AML

Treatment-resistant; needs immediate transplant

6

Poor Matching Results

No suitable donors identified

7

Non-Malignant Conditions

E.g., severe aplastic anemia