2024 CIBMTR Report of Survival Statistics for Blood and Marrow Transplants

September 12, 2025

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Tables

Abbreviations

Abbreviation	Expansion
ALL	acute lymphoblastic leukemia
alloHCT	allogeneic hematopoietic cell transplantation
AML	acute myeloid leukemia
autoHCT	autologous hematopoietic cell transplantation
BEAM	BCNU [carmustine], etoposide, Ara-C [cytarabine], and melphalan
Bu	busulfan
Carb	carboplatin
CBV	cyclophosphamide, BCNU [carmustine], and VP-16 [etoposide]
CML	chronic myeloid leukemia
CNI	calcineurin inhibitor
CNS	central nervous system
CR	complete remission
CR ≥ 2	second or greater complete remission
CR1	first complete remission
Cy	cyclophosphamide
Dx	diagnosis
Etop	etoposide
FA	Fanconi anemia
FCR	fludarabine, cyclophosphamide and rituximab
Flu	fludarabine
GVHD	graft-versus-host disease
HCT	hematopoietic cell transplantation
HL	Hodgkin lymphoma
HLA	human leukocyte antigen
MDS	myelodysplastic syndrome
Mel	melphalan
MM	multiple myeloma
MMF	mycophenolate mofetil
MTX	methotrexate
NE	not evaluable
NHL	non-Hodgkin lymphoma
NMA	non-myeloablative
PR	partial remission
PTCy	post-transplant cyclophosphamide
RIC	reduced-intensity conditioning
SAA	severe aplastic anemia
SCID	severe combined immunodeficiency
TBI	total body irradiation
TLI	total lymphoid irradiation
TT	thiotepa
VP	etoposide

Introduction

The following tables describe the use and outcome of autologous and allogeneic blood and bone marrow transplants in the more than 350 centers participating in CIBMTR® (Center for International Blood and Marrow Transplant Research®). Prior to 2007, we estimate CIBMTR captured 90% of all unrelated donor transplants performed in the US, 60-90% of related donor allogeneic transplants, and 65-75% of autologous transplants. After 2007, CIBMTR collects data on all allogeneic transplants performed in the US and 80% of autologous transplants.

Table 1-13 (autologous) and Tables 14-36 (allogeneic) show patient characteristics and probabilities of survival (±95% CIs) post-transplant at 100 days, and at 1, 3 and 5 years. Categorical variables are represented by N (%), continuous variables by median (range). Probabilities were calculated using the Kaplan-Meier estimator. Some groups lack sufficient data for calculation of probabilities beyond 2-3 years. These are indicated by footnotes which give the time of the last censored observation. Outcomes were stratified on disease and disease state pre-transplant. However, it should be remembered that these groups are still heterogeneous with regard to age, prior treatment, chemotherapy-sensitivity and other important prognostic factors. Extrapolating to individual patients or centers may not be appropriate.

The enclosed raw data represents a preliminary review of information registered to CIBMTR. The analysis has not been reviewed or approved by the Advisory or Scientific Committee of CIBMTR.

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Data source

the Medical College of Wisconsin and NMDP. CIBMTR collaborates with scientists around the globe to study cellular therapies and cure life-threatening diseases, many of which are rare.

More than 310 medical centers worldwide submit clinical data to CIBMTR about hematopoietic cell transplantation and other cellular therapies, such as chimeric antigen receptor T cells (CAR-Ts). These therapies treat malignant and non-malignant hematologic conditions and some other disorders. By analyzing these data, CIBMTR identifies the best treatments to help people live longer and enjoy better quality of life.

CIBMTR also collects and analyzes participant-reported outcomes and quality of life data.

CIBMTR maintains one of the world's largest observational databases of clinical information on HCT and other cellular therapies. Data are collected, stored, and shared under terms of the *Protocol for a Research Database for Hematopoietic Cell Transplantation, Other Cellular Therapies and Marrow Toxic Injuries* and its accompanying consent forms.[1-5] At the time of treatment, patients consent for their data to be entered into the Research Database and used to support a broad research agenda. Currently, CIBMTR's Research Database includes long-term clinical data from more than 700,000 patients.

The database includes:

- Almost 100% of allogeneic transplants in the US
- More than 85% of autologous transplants in the US
- About 27,500 patients who received other cellular therapies

CIBMTR holds the contract for the Stem Cell Therapeutic Outcomes Database, awarded by the Health Resources and Services Administration of the US Department of Health and Human Services. As the contract holder, CIBMTR is charged with collecting data on all allogeneic (related and unrelated) hematopoietic cell transplantations performed in the US. All US transplant centers are required to report data to CIBMTR; participation of non-US centers is voluntary.

CIBMTR also maintains a biorepository with tissue samples.[5] CIBMTR's unique staff of physicians, biostatisticians, research coordinators, data management staff and IT teams conduct both observational research and clinical trials.

Transplant data

CIBMTR collects transplant data on 2 levels: Transplant Essential Data (TED) and Comprehensive Report Form (CRF). CIBMTR collects TED data on all patients. TED data are an internationally accepted standard data set that includes hundreds of details about patients' demographics, disease, treatment, response, side effects, and long-term outcomes. Using a regularly reviewed, weighted algorithm, CIBMTR selects a subset of patients for more detailed CRF data collection.

Approximately 75% of CIBMTR centers provide CRF data; this accounts for more than 25% of cases submitted to CIBMTR annually.

TED and CRF data are collected pre-transplant, 100 days post-transplant, 6 months post-transplant, annually until year 6 post-transplant, and biannually thereafter until death or lost to follow-up.

Data quality

CIBMTR subjects data to a series of automated and manual quality checks. CIBMTR data operations teams work directly with centers to maintain both consistency and quality of data collected. In addition, CIBMTR performs on-site source data audits, and each member center is audited within a 4-year cycle. These validations and verifications produce high-quality data. If a center fails to meet data quality standards, its data are removed (embargoed) from research studies.

Ethics

CIBMTR protects the privacy and human rights of participants.[6] CIBMTR obeys international laws and ethical guidelines, including the United States Health Insurance Portability and Accountability Act (HIPAA) and the European Union's General Data Protection Regulation (GDPR). The NMDP Institutional Review Board, which is fully accredited by the Association for the Accreditation of Human Research Protection Programs, reviews CIBMTR's research. Patients and/or guardian(s) give informed consent for research.

Results

Note: The enclosed data are confidential and represent a preliminary review of information submitted to the CIBMTR. The analysis has not been reviewed or approved by the Statistical or Scientific Committees of the CIBMTR. The data may not be published without prior approval of the CIBMTR.