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Perspective

# Current Use and Trends in Hematopoietic Cell Transplantation in the United States

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A B S T R A C T

Hematopoietic Cell Transplantation (HCT) is an established curative treatment for a number of malignant and nonmalignant diseases involving the hematopoietic system and some solid tumors. In this report, we provide information about the number of HCT procedures performed in the United States in 2015, and analyze trends and outcomes of HCT as reported to the Center for International Blood and Marrow Transplant Research (CIBMTR). We show that the number of HCTs performed annually continues to increase, as the indications for HCT, preferred donor sources, and graft-versus-host prophylaxis continue to evolve. We report on general overall survival by indication, by disease status at transplantation, and by transplant type. This report provides a current perspective on transplantation activity in the United States with a focus on recent trends in alternative donors and contemporary transplantation practices.



## INTRODUCTION

Hematopoietic Cell Transplantation (HCT) is an established curative treatment for a number of conditions, including malignant hematologic diseases and nonmalignant congenital and acquired diseases involving the hematopoietic system. More than 1 million HCTs have been reported worldwide over the last 6 decades [1]. In this article, we report and analyze current trends and outcomes of HCT in the United States as reported to the Center for International Blood and Marrow Transplant Research® (CIBMTR®) are described and further discussed.

The CIBMTR began as the International Bone Marrow Transplant Registry (IBMTR) in 1972. The Autologous Bone Marrow Transplant Registry (ABMTR) was started in 1989. When the IBMTR and the ABMTR merged with the National Marrow Donor Program Database in 2004, the CIBMTR was formed. The CIBMTR database includes data reported by more than 500 centers in 54 countries worldwide, including 205 active US centers. More than 227,906 autologous, 196,209 allogeneic related and unrelated donor (URD), and 11,225 cord blood transplantation procedures have been reported through 2015. Starting in 2007, the C.W. Bill Young Stem Cell Act required that all allogeneic procedures performed in the United States be reported to the CIBMTR. Autologous procedures in the United States are still reported voluntarily, and currently the CIBMTR captures 75% to 80% of US autologous transplant activity. Outcomes data are collected prospectively.

## METHODS

### *Data Collection*

Transplantation centers collect and submit data on their transplant populations to the CIBMTR, which centrally assigns the data to either a Transplant Essential Data (TED) track that collects minimal data or a Comprehensive Report Form (CRF) track that entails more detailed data collection. Data collection time points include pretransplantation and 100 days, 6 months, and 1 year post-transplantation, then annually until death. Repeat cellular infusions from the same donor and second transplantations are captured as well. All data are entered electronically into the FormsNet system, which enforces allowable data and performs simple logical checking. Centers are audited once within a 4-year audit cycle, during which data submitted to the research database are compared with source documents. Discrepancies are reviewed, and centers may be required to submit a corrective action plan following an audit. Data from allogeneic recipients are used for the Center-Specific Outcomes Analysis, an annual report federally mandated for all transplantations performed in the United States by the C. W. Bill Young Act of 2007. In this report, we summarize key transplantation activity based on data submitted to the CIBMTR in 2015. This summary is also available in part online [2].

### *Statistics*

Total transplantation numbers are estimates based on data reported to the CIBMTR on both TED forms and CRFs. Overall survival probabilities are presented according to disease, disease status, donor type, year of

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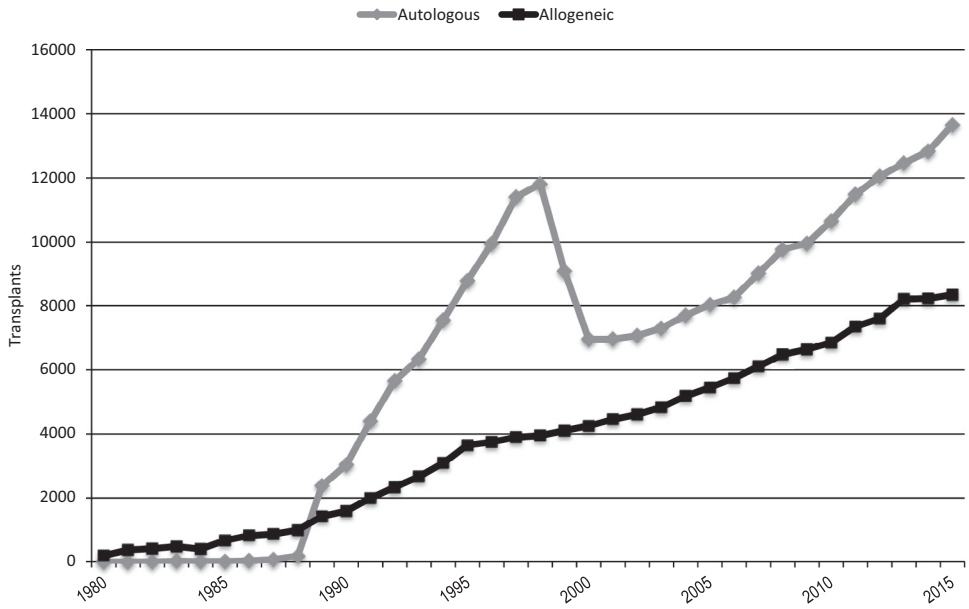
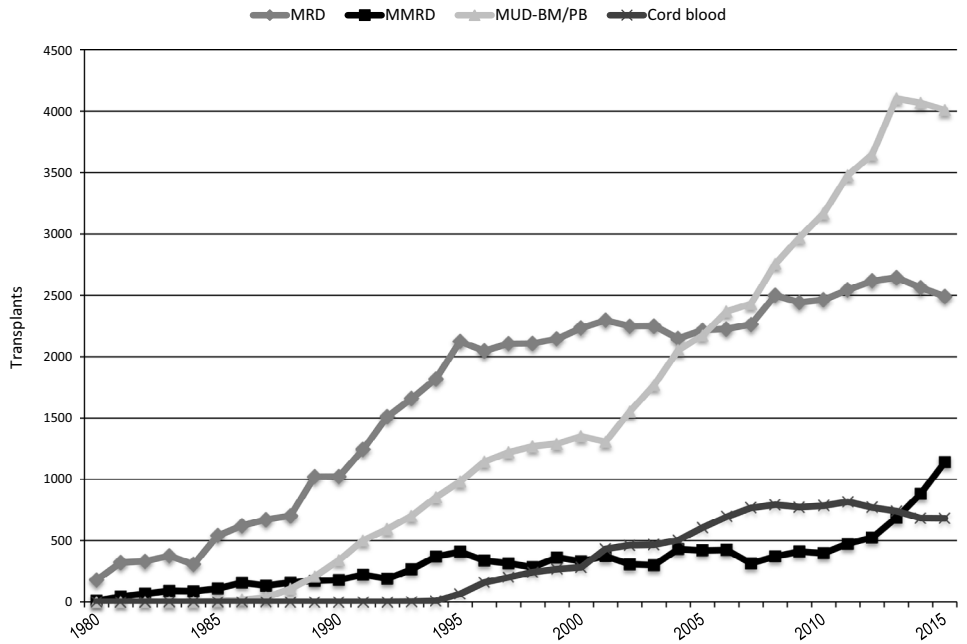



**Figure 1.** Estimated annual number of HCTs performed in the United States.

transplantation, and conditioning regimen intensity. Comparisons across survival curves are univariate and are not adjusted for potentially important contributing factors. Causes of death are as reported by centers. This analysis focuses on trends in transplantations performed in the United States from 1980 to 2015. Survival analysis using the Kaplan–Meier estimator was performed and is reported in a 3-year time point with standard error. Assessment of survival was not adjusted for other significant covariates associated with survival.

*Transplantation Activity in the United States*

Figure 1 shows the estimated annual numbers of transplantations performed in the United States. In 2015, 13,658 autologous, 2351 matched related donor, 3810 adult URD, 649 cord blood, and 653 haploidentical transplantations were performed. The number of autologous HCTs has increased steadily since 2000, mainly for the treatment of plasma cell and lymphoproliferative disorders. There has been a significant trend toward increasing use of autologous HCT over the last few years, owing to an increase in older recipients and recipients with lymphoproliferative diseases. The use of allogeneic HCT with a URD has increased steadily (although it has leveled off in the last few years), and has surpassed related donor HCT since 2006 (Figure 2). Major factors contributing to this trend included the growth of URD registries and cord blood inventories, improved URD transplantation outcomes, and increased use of allogeneic HCT in patients age >60 years with reduced-intensity conditioning.




Legend: MRD- matched related donor; MMRD- mismatched related donor; MUD-BM/PB- matched unrelated donor-bone marrow/peripheral blood

**Figure 2.** Allogeneic HCTs in the United States by donor type.