

PROJECT SUMMARY/ABSTRACT

This project aims to leverage the comprehensive research database established by the Center for International Blood and Marrow Transplant Research® (CIBMTR) for hematopoietic cell transplantation (HCT). The project is divided into three specific aims: a descriptive analysis of enrolled patients to understand demographic and clinical characteristics, a survival analysis focusing on the time from HCT to seven different endpoints, and the development of an R Shiny application for dynamic and interactive visualization of study results. This initiative seeks to enhance understanding of HCT outcomes, identify factors influencing survival post-transplant, and facilitate data accessibility for clinicians and researchers alike, ultimately contributing to improved patient care and outcomes in the field of cellular therapies.

SPECIFIC AIMS

At the highest level, what is the area the study focuses on?

The study focuses on hematopoietic cell transplantation (HCT). It aims to analyze the outcomes, survival rates, and other relevant data of patients enrolled in the CIBMTR research database. This area is critical for advancing the understanding and effectiveness of HCT in treating sickle cell diseases, with the overarching goal of improving patient outcomes and the success of this treatment.

What have I done that is relevant to the study?

- clinical research
- data management
- statistical analysis
- develop R Shiny application

What are the hypotheses the initial work has generated, which will be the focus of the study.

Specific Aims:

- Descriptive Analysis of Enrolled Patients
- Survival Analysis
- Development of an R Shiny Application for Results Visualization

What comes next? What will the impact be of achieving the specific aims?

- Clinical Practice Implications: Findings could inform clinical practice by identifying key factors affecting patient outcomes, potentially leading to updated guidelines or strategies for patient management post-HCT.
- Future Research Directions: The project may highlight gaps in the current knowledge or suggest hypotheses for future studies, thereby guiding subsequent research efforts in HCT and cellular therapy.

RESEARCH STRATEGY

A. Significance

Reintroduce the domain.

The domain of Hematopoietic Cell Transplantation (HCT) represents a pivotal area in the treatment of a variety of hematologic malignancies, immune deficiencies, and certain non-malignant disorders. These therapies offer potentially curative treatments for patients with conditions such as leukemia, lymphoma, myeloma, and sickle cell disease, among others. The complexity of HCT processes, including donor selection, conditioning regimens, and post-transplant care, necessitates ongoing research to optimize patient outcomes and minimize complications.

What is the impact/significance of what is done now?

- Improving Patient Outcomes: By conducting a comprehensive analysis of patient data, this study aims to uncover patterns and predictors of success in HCT and cellular therapies. Identifying factors that influence outcomes such as survival rates, complication rates, and quality of life post-transplant can lead to more personalized and effective treatment strategies.
- Innovative Data Visualization: The development of an R Shiny application for visualizing the results offers a novel tool for clinicians and researchers. This interactive platform will facilitate a deeper understanding of the data, enabling users to explore the impact of various factors on patient outcomes dynamically.

What does the study do?

- Bridges the Gap Between Data and Practice: By integrating descriptive and survival analyses with advanced data visualization techniques, the study bridges the gap between raw clinical data and actionable insights. It provides a user-friendly interface for exploring complex datasets, making it easier to derive meaningful conclusions that can inform clinical decisions.
- Facilitates Evidence-Based Decisions: The study empowers clinicians and researchers to make evidence-based decisions regarding HCT and cellular therapy practices. By providing access to comprehensive analyses and interactive tools, it supports a more nuanced understanding of how different variables affect patient outcomes.

How will this change research in the domain?

Promoting Personalized Medicine: The insights gained from this study could advance the field toward more personalized medicine approaches in HCT and cellular therapies. Understanding the specific factors that influence individual patient outcomes can lead to more tailored and effective treatment plans, ultimately improving the overall success rate of these therapies.

Important significance topic.

Another important significance topic.

B. Innovation

What is different/new/novel about this project?

- **Comprehensive Integration of Descriptive and Survival Analyses:** While many studies focus on either descriptive analyses of patient demographics or survival analyses separately, this project innovatively combines both. This dual approach allows for a more nuanced understanding of how specific patient characteristics impact outcomes post-transplantation.
- **Development of an Interactive R Shiny Application for Data Visualization:**
 - **Dynamic, User-Driven Exploration:** Unlike static reports or publications, the R Shiny application allows users to interact with the data in real-time, adjusting parameters to explore different scenarios and outcomes. This level of interactivity is novel in the field of HCT research.
 - **Accessibility to Non-Statisticians:** By providing a user-friendly interface, the application makes complex statistical analyses accessible to clinicians, researchers, and potentially patients who may not have expertise in data analysis. This democratization of data is a significant innovation.

C. Approach

Background - what are the existing concepts this study will use?

- **Hematopoietic Cell Transplantation (HCT) and Cellular Therapies:**
 - **Clinical and Demographic Data Analysis:** Utilizing patient data from HCT and other cellular therapies, including information on donor types, conditioning regimens, and transplant outcomes.
 - **Survival and Outcome Metrics:** Focusing on metrics like overall survival, disease-free survival, incidence of GVHD, relapse rates, and other complications post-transplant.
- **Statistical Methods for Survival Analysis:**
 - **Kaplan-Meier Estimates:** To calculate survival probabilities over time for various patient cohorts.
 - **Cox Proportional Hazards Model:** To identify the factors that significantly influence patient outcomes, adjusting for covariates.
- **Data Visualization and Interactive Tools:**
 - **R Shiny:** Development of interactive web applications for dynamic data exploration. R Shiny applications enable users to interact with the data in real-time, adjusting parameters to visualize different outcomes or demographic analyses.

How will they be put together?

- **Data Preparation and Initial Analysis:** Gathering and cleaning the data from the CIBMTR research database, followed by descriptive analyses to understand the patient population and treatment characteristics.
- **Deep-Dive into Survival Analysis:** Utilizing the identified variables from the descriptive analysis, conducting survival analysis to explore the relationships between patient characteristics, treatment variables, and outcomes.
- **Development and Deployment of the R Shiny Application:** Integrating the insights from both descriptive and survival analyses into an interactive tool. This application will not only showcase the results but also allow for the exploration of hypothetical scenarios, such as the impact of different donor types or conditioning regimens on patient outcomes.

D. Specific Aim 1: Descriptive Analysis of Enrolled Patients

This aim involves a detailed examination of the demographic and clinical characteristics of patients who have undergone HCT and other cellular therapies. By analyzing factors such as age, gender, diagnosis, type of transplant, and donor characteristics, we aim to identify patterns and trends that could inform future patient care strategies.

E. Specific Aim 2: Survival Analysis

We will perform survival analysis to assess the time from HCT to various endpoints, including graft-versus-host disease (GVHD), relapse, infection, and overall survival. This analysis will help identify key predictors of outcomes and potentially modifiable factors that could improve patient survival and quality of life.

F. Specific Aim 3: Development of an R Shiny Application for Results Visualization

To make the findings accessible and actionable, an R Shiny application will be developed. This interactive tool will allow users to explore the data dynamically, visualize survival curves, compare outcomes across different patient groups, and potentially identify new areas for research or intervention.

Hypothesis: What is being tested? Rationale: Why is this something we should be testing? Experimental Approach: How is the test going to be implemented? Interpretation of Results: How does the test translate to science? Potential Problems and Alternative Approaches: What will we do if it doesn't work?

Note: Material below is for reference.

F.1. Overview of the proposal

F.2. Research team

F.3. Preliminary studies

F.4. Resources

F.5. Design and methods for Aim 1

F.6. Design and methods for Aim 2

F.7. Design and methods for Aim 3

F.8. Timeline

There are lots of good examples of R-based Gantt charts to be found by clever Googling. For displaying progress with sidebar annotations by aim, I particularly like [this](#) example from the `lares` package.

F.9. Rigor and reproducibility

F.10. Impact of the proposed study

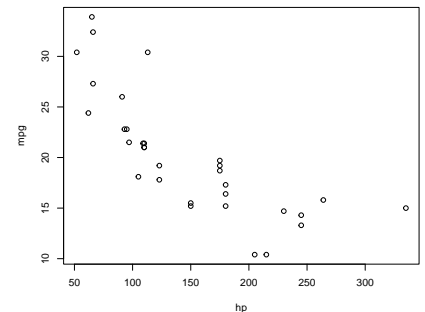


Figure 1: Important scatterplot