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Guest editor introduction to issue on transplant statistics

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This special issue focus on statistical problems motivated by the analysis of data from hematopoietic stem cell transplantation (HSCT) also known as bone marrow transplantation. This type of therapy has been used for a number of years as a treatment for leukemia and lymphoma; for solid organ cancers; for immune deficiency disorders and more recently for autoimmune disorders. The clinical course of a transplant patient is often quite complex involving the analysis of many competing risks such as relapse and death in remission; or multistate models of disease such as engraftment, graft-versus-host disease, relapse, recovery from relapse, second relapse, and death. Potential risk factors for modeling treatment success can be simple fixed covariates such as race, gender or quite complicated time varying covariates such as post transplant therapies or post transplant performance status.

While the analysis of hematopoietic stem cell transplant is challenging, many of the problems encountered in the analysis of HSCT are found in the analysis of other lifetime data problems. The methods in this special issue, while illustrated on HSCT data, are generalizable to the wider range of applications.

The first paper in the edition is by Eapen and Rocha. This paper by two transplant physicians provides medical background in Hematopoietic Stem Cell Transplantation. A basic understanding of the medical background of a problem is critical for understanding how to apply the techniques of lifetime data analysis to a problem and this paper provides that background for the HSCT problem.

Multistate models arise in many analyses of HSCT data. These models may be simple competing risks problems like studies of relapse and death in remission; they may involve the inter relationship between engraftment, complications of the transplant such as infection or graft versus host disease and primary events such as death or relapse; or they may involve the study of post transplant therapies applied to patients who experience some post transplant event. Three papers in this issue deal with these types of problems. Andersen and Perme give a review of multistate model techniques based on either transition intensity models or on direct estimation of transition or state occupation probabilities. Putter and van Houwelingen compare a new landmark analysis to traditional multi-state model analysis. Liu, Logan and Klein look at inference for “current leukemia free survival” which is the chance a patient is alive and in remission (first or subsequent) after transplant.

Three additional papers deal with problems arising in a regression analysis of HSCT data. Scheike and Zhang consider regression models for the cumulative incidence function that generalize the work of Fine and Gray (*JASA* V 94, 1999) and they provide goodness of fit tests for their models. Storer, Golley and Jones consider the problem of using a regression model

to provide an estimate of survival probabilities adjusted for a set of covariate values. Ibrahim, Chen and Kim look at the problem of variable selection when some of the patients have missing variable information in a Cox model. They approach the problem from a Bayesian prospective.

The final paper by Logan and co-workers discusses the statistical approach used by the National Marrow Donor Program to provide a report card on patient survival probability to each transplant center that they facilitated transplants for. Based on recent legislation this method is proposed to expand the report to each US center performing any type of allogenic HSCT.

Most of the papers in this issue use data provided by the Center for International Blood and Marrow transplantation (CIBMTR). This is an international group of transplant centers who provide data on all consecutive transplants to a central data coordinating center. This center is housed in the Medical College of Wisconsin in Milwaukee and at the National Marrow Donor Program in Minneapolis. The center performs retrospective studies on the world's largest blood and marrow transplant databases and tissue sample repositories to identify the most promising transplant approaches and the patients most likely to benefit from this therapy. It designs and implements clinical trials related to questions arising in HSCT. It makes available research resources to investigators including the world's largest clinical database of related blood and marrow transplants, along with repositories of thousands of matched tissue samples from transplant recipients and their donors—including significant numbers of samples for many rare diseases. The CIBMTR actively supports research into biostatistical methods directed at problems in HSCT. Researchers can propose studies to the CIBMTR or request data from the registry for their own investigations. Details on that service are found at the CIBMTR web site at <http://www.cibmtr.org>.

The activities of the CIBMTR are sponsored under a U24 grant from National Institutes of Health/National Cancer Institute (CA76518) and by a contract from Health Resources and Services Administration (HRSA). The later contract requires that all HSCTs performed in the US be reported to the CIBMTR. We are both supported by these projects and by a grant from the National Cancer Institute (R01 CA54706-13).