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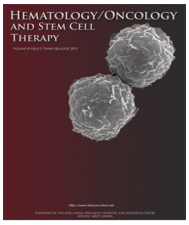

Current concepts on hematopoietic stem cell transplantation outcome registries; Emphases on resource requirements for new registries

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Abstract
There is tremendous variability in size, scope, and resource requirements for registries depending on the number of patients and participating sites. The outcome registries are organized systems to collect uniform data using an observational study methodology. Patient registries are used to determine specified outcomes for a population for predetermined scientific, clinical, or policy purposes. Historically, outcome registries established in the development of hematopoietic stem cell transplantation (HSCT) have now evolved into myriads of locoregional and international transplant activity and outcome resources. Over time, these registries have contributed immensely in determining trends, patterns, and treatment outcomes in HSCT. There is wider variation in the goals, mission, objectives, and outcomes of the ongoing registries depending on the organizational structure. There is a growing trend toward overarching relationship of these registries to serve as complementary and interoperable resources for high potential collaborative research. In addition to capacity building, standardized, accredited, and optimally operational registries can provide unmatched and unparalleled research data that cannot be obtained otherwise. Moving forward, HSCT data collection, collation, and interpretation should be an integral part of the treatment rather than an option. Quality assurance and continuous quality improvement of the data are pivotal for credibility, measurable/quantifiable outcomes, clinically significant impact, and setting new benchmarks.

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Introduction

Observational data are a significant research tool in determining current trends, patterns of care, short- and long-term follow-up, and outcome analysis for adverse events and effectiveness. The outcome registries are the established systems for data collection to assess specific outcomes for particular diseases, conditions, exposures, interventions, modalities, or socioeconomic status to fill in the knowledge gaps. These measurable and quantifiable analyses and outcomes also serve as the bases for future studies, resource allocation, planning, and policy improvement [1—4]. Registries are classified according to population and purpose. Populations may be defined by disease category, exposures (interventions, treatments, procedures, adverse events), or groups (geography, socioeconomic status, and others). Registries are most commonly aimed at the natural history of disease, treatment effectiveness, toxicity/safety, and quality assessment, in a methodical approach [5].

Historically, outcomes registries have been instituted very early in the development of innovative treatments including hematopoietic stem cell transplantation (HSCT) for multitudes of malignancies and life-threatening disorders. The source documents for these observational outcomes registries are mostly the patient's medical record. These registries foster research into outcomes by focusing on questions difficult to answer by clinical trials and harmonizing clinical trial outcomes. Registries provide the state of current knowledge and gaps in evidence to form the basis for prevention/intervention program development, delivery, and effectiveness. They can help in designing the optimal schema for prospective and retrospective studies and for comparative analyses of diverse HSCT strategies or for HSCT versus non-HSCT therapies. Registries are particularly useful in situations where a comprehensive and flexible research design is needed or when the purpose is to discover how a product works in a wide variety of subgroups including ethnicity and socioeconomic status [6—9]. A hybrid approach registry collects data retrospectively and prospectively. If data collection is sufficiently comprehensive, outcomes findings from patient registries can be widely generalizable (see Tables 1—3).

Outcome registries can synergize continuous quality improvement processes by data feedback and reporting loops to optimize patient care with better understanding of complications. These registries offer insight of patient population, demographics, clinical epidemiology [10,11], changing patterns of care, rates of complications, and adverse events [12—14]. The value of HSCT registries is augmented by following established ethical and quality standards for the design, collection, analysis, reporting, monitoring, and auditing of the registry data. Following a good registry practices can strengthen scientific rigor and transparency of the data. Easy accessibility of registry data to physicians and safeguards for credible, accurate, and reliable data are cardinal elements of a quality registry [15]. Registries must provide assurance for the confidentiality and integrity of data.

Regional HSCT registries can promote HSCT in a specific region, identify locoregional trends and practices, standards and interventions, and may also be helpful in benchmarking HSCT outcomes [16—19]. National registries can be used to benchmark transplant outcome using the large multinational outcomes registry [European Group for Blood and Marrow Transplantation (EBMT), Center for International Blood and Marrow Transplant Research (CIBMTR), Eastern Mediterranean Bone Marrow Transplantation (EMBMT)] as a Ref. [20] The CIBMTR conducts annual assessment of 1-year survival post-allogeneic HSCTs in each transplant center in the United States and provides it to participating centers and the public [21,22]. Globalization of patient and donor registration for HSCT is a realistic goal and can contribute to the improvement of patient care, outcomes, and donor safety [23,24]. Registry data have provided important insights into international differences in indications for HSCT, and access to HSCT [25,26]. Data from outcomes registries can help to discern regional and international outcome variations to identify modifiable practices for process improvement. Properly designed, maintained, and analyzed observational registries can provide invaluable information and advantages. The availability of detailed clinical information from registries can provide timely, accurate, and reliable evidence on the safety and efficacy of HSCT [27—32] in myriads of hematological malignancies and rare acquired and inherited hematologic conditions [33—40].

Transplant registries are simple way of data collection. However, they face number of challenges as summarized below in terms of trained personnel, rules and regulations,

Table 1 Strengths of registry based research.

- Comprehensive registry; excellent source of demographic and activity data — dynamic measure of patterns of care
- Useful for planning intervention trials — hypothesis generation, calculating effect size and potential recruitment
- ''Real world" therapeutic effectiveness and safety data (as opposed to efficacy) — compare disease management by program, region, country
- Heterogeneity of standard practice across participating sites facilitates research into 'best practices'
- Heterogeneity among study subjects
- Detection of rare consequences is satisfied by large numbers of patients followed for long periods of time — a unique advantage
- Low risk to participating subjects (observational rather than interventional) can promote broad participation
- Flexibility: Serves as a platform for extending observation or intervention to particular groups of subjects — sub-studies
- Relatively low cost to develop and maintain on a per-patient basis
- Useful as a comparative arm in comparative effectiveness research
- Provide meaningful data for decision-making where a clinical trial is not feasible or practical
- Approximation of treatment impacts are more realistic

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