Biol Blood Marrow Transplant 23 (2017) 717–725

Biology of Blood and
Marrow Transplantation

journal homepage: www.bbmt.org

Reports

National Institutes of Health Blood and Marrow Transplant Late Effects Initiative: The Healthcare Delivery Working Group Report

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Article history:
Received 24 September 2016
Accepted 30 September 2016

Key Words:
National Institutes of Health
Late Effects Consensus
Conference
Hematopoietic cell
transplantation
Survivorship
Care delivery models
Healthcare value

A B S T R A C T

Hematopoietic cell transplantation (HCT) survivors are at risk for development of late complications and require lifelong monitoring for screening and prevention of late effects. There is an increasing appreciation of the issues related to healthcare delivery and coverage faced by HCT survivors. The 2016 National Institutes of Health Blood and Marrow Transplant Late Effects Initiative included an international and broadly representative Healthcare Delivery Working Group that was tasked with identifying research gaps pertaining to healthcare delivery and to identify initiatives that may yield a better understanding of the long-term value and costs of care for HCT survivors. There is a paucity of literature in this area. Critical areas in need of research include pilot studies of novel and information technology supported models of care delivery and coverage for HCT survivors along with development and validation of instruments that capture patient-reported outcomes. Investment in infrastructure to support this research, such as linkage of databases including electronic health records and routine inclusion of endpoints that will inform analyses focused around care delivery and coverage, is required.

INTRODUCTION

The number of hematopoietic cell transplantation (HCT) survivors in the United States is projected to surpass half a million by 2030 [1]. HCT survivors continue to be at risk for late complications, and lifelong follow-up for screening and prevention is recommended [2,3]. Similar to the principles of cancer survivorship care [4-6], care for HCT survivors should include surveillance for cancer recurrence and new cancers, prevention of subsequent cancers and late effects, interventions for other illnesses caused by cancer and its treatment, and coordination of care among healthcare providers to ensure that all health needs of survivors are met. Several healthcare delivery models have been offered for the longitudinal care of cancer survivors [7-10]. However, the applicability of these survivorship models to HCT recipients has not been evaluated. HCT is a specialized procedure, and transplant

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Financial disclosure: See Acknowledgments on page 724.

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http://dx.doi.org/10.1016/j.bbmt.2016.09.025

survivors have unique treatment exposures, risk factors, and healthcare needs that are likely best addressed through patient-centered, integrated health delivery models that are specific for HCT [4,5,11].

Economic considerations are important determinants of healthcare delivery, and issues around costs and resource utilization at the patient, provider, institutional, and societal levels need to be addressed within the healthcare delivery model for HCT survivors. Successful HCT may decrease healthcare utilization, as has been reported in patients with sickle cell disease, as compared with control subjects who did not undergo transplantation [12]. Although quality of care is the fundamental goal of long-term management of patients, value of care is equally important for sustainability of the healthcare system. "Value," which in simple terms is the ratio of quality over cost, can integrate other healthcare goals such as safety, patient-centeredness, efficiency, cost-containment, equity, and access [13]. There is increasing recognition of the need to better address other elements of value-based care among the HCT recipient population (eg, improved data collection/reporting, use of innovative technologic tools, evidence-based practice supported by clinical trials, and reduction of variation to facilitate these elements) [14].

Healthcare Delivery Working Group

The Healthcare Delivery Working Group (HCD-WG) is 1 of 6 working groups established in June 2015 as part of the National Institutes of Health (NIH) Blood and Marrow Transplant Late Effects Initiative. Sponsored by the National Cancer Institute and National Heart, Lung and Blood Institute, it is a collaborative multistakeholder effort focused on developing research agendas to improve transplant survivor outcomes. The working groups met via regular conference calls and convened for a final symposium in June 2016. The HCD-WG was assigned the task of identifying research gaps pertaining to healthcare delivery and to identify initiatives that may yield a better understanding of the long-term value and costs of care for HCT survivors. The group had representation from transplant center medical directors, payers, NIH, Health Resources and Services Administration, health services researchers, and allied health practitioners from the United States, Canada, and Europe. Members also represented stakeholder professional organizations, including the American Society for Blood and Marrow Transplantation, the European Group for Blood and Marrow Transplantation, and the Center for International Blood and Marrow Transplant Research (CIBMTR), and patient advocacy groups.

For the purposes of this overall effort, HCT survivors were defined as pediatric or adult, autologous or allogeneic HCT recipients who had survived for 1 year or longer after their transplant. From a care delivery model and coverage perspective, the HCT episode can be divided into several phases [15,16]. The first is the evaluation phase, during which services required to assess and evaluate whether a patient and in the case of allogeneic HCT the donor are suitable for the transplantation procedure. Second, the pretransplant care phase includes care provided from the time a patient is identified as a candidate for HCT and includes all related care until the initiation of conditioning regimen. The transplant phase, the third, phase, usually begins with the start of the conditioning regimen and can last 30 to 120 days post-transplant; this phase covers stem cell infusion and transplant hospitalization and often includes graft procurement, stem cell mobilization, and processing. The fourth phase is follow-up care phase, which starts on completion of the transplant phase and can extend until the patient is discharged from routine transplant center follow-up care. Survivorship care is considered to be part of this last phase. In this article we provide background and a summary of pertinent issues, priorities, and opportunities for future research relevant to healthcare delivery for HCT survivors.

HEALTHCARE DELIVERY MODELS

Conceptual Framework and Methodology

To identify the essential components of survivorship care, the HCD-WG considered several overlapping perspectives with emphasis on individualizing survivorship care for HCT recipients (Figure 1). First, emphasis was placed on both the whole patient–family unit impacted by the HCT survivorship experience. Second, it was determined that patient-centered survivorship care should be responsive to patient healthcare needs and preferences. Third, models should be developed with the specific considerations of the HCT care experience in mind; for example, the care delivery model may need to consider the distance from the transplant center, active transplant-specific complications such as graft-versus-host disease (GVHD), and availability of local providers who are willing to learn and take care of medical issues in partnership with the transplant team. Fourth, care models need to be dynamic and adaptable because patient status and healthcare needs can change over time (eg, disease recurrence, onset or resolution of GVHD, and occurrence of infections or late complications), with a keen focus on both the economic environment and the payer landscape. Responding to this continuum of care necessitates coordination between the transplant center and local providers, including hematologist-oncologists, specialists, and primary care providers. Fifth, survivorship care should be evidence-based, high quality, and individualized to patient-specific exposures and risk factors from the pre- and post-transplant period. Finally, care delivery models must account for the variability of resources and personnel available at transplant centers [17,18] and at community practices and consider use of innovative care delivery methods that integrate technology.

Stakeholders in HCT survivorship

In addition to the patients and families, stakeholders in HCT survivorship care include transplant centers and referring hematology-oncology and primary care providers. We acknowledge the increasing importance of nonphysician providers in the care of HCT survivors (eg, advanced practice professionals, nurses, social workers, and pharmacists). HCT patients regularly receive fragmented care as they move between community providers and the transplant center, which are most often located in academic health centers. In this mix of care delivery settings, the payer may be the only common link between providers and can play an important

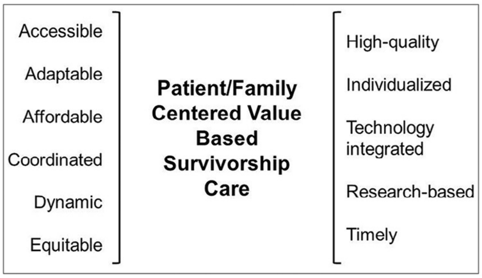

Figure 1. Conceptual model showing the essential elements of survivorship care for HCT recipients.