



# Biology of Blood and Marrow Transplantation

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## Editorial

### Economic Challenges in Hematopoietic Cell Transplantation: How Will New and Established Programs Face the Growing Costs?



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#### Article history:

Received 23 May 2017

Accepted 28 July 2017

The life-saving advances over the 60-year history of hematopoietic cell transplantation (HCT) have been truly astounding. The Worldwide Network for Blood and Marrow Transplantation (WBMT) is committed to making safe and effective HCT available to all in the global population who could benefit from it [1]. In resource-limited areas of the world, we need to emphasize how focused, thoughtful care models can treat those with standard HCT indications and save many lives. However, with the new genomics, engineered cellular products, anti-infectives, and remarkable progress in targeted therapies [2], patients come to transplantation with a lower disease burden, a better understanding of their disease phenotype, and an expectation of better outcomes. Additional characterization of patient risks based on comorbidities, frailty, and tailored approaches to necessary supportive care have allowed expansion of HCT to an older and sicker population. This also extends the potential of transplantation therapy to help many more patients, particularly those in the age

group in which transplantation-targetable diseases are most prevalent.

All of these advances come with added clinical complexities and added costs, however. The high price tags of the many new pharmaceuticals, small molecules, and biologicals are daunting, but the prices charged and reimbursement paid should properly be addressed in health policy debates. The financial burden is also growing due to newly developed diagnostic testing, including molecular measures of residual disease, and newly recognized microbiological and virological pathogens. In addition, costly supportive care therapies, such as those for veno-occlusive disease, thrombotic microangiopathies, and other life-threatening toxicities, are contributing to this burden. These can all markedly escalate the financial burden facing patients, transplantation centers, and payers when delivering the life-saving promise of HCT.

#### LEARNING TO DO MORE WITH LESS

Can we, as a field, learn to define best practices for safe and cost-efficient care? Can we, in the spirit of choosing wisely [3], define minimal required standards for laboratory monitoring, transfusion support, disease burden assessment, and follow-up care? In addition, for newly developing programs, particularly in resource-limited regions of the world, establishing safe and well-planned core elements of HCT support can allow many patients to benefit from the curative potential of HCT within affordable limits. We have to ask whether we will continue to adopt new complex and quite costly diagnostics and therapeutics, which are intellectually satisfying but may add only minimal safety or clinical value to our patients?

To begin this discussion, WBMT led a joint international session at the BMT Tandem Meetings in February 2017

*Financial disclosure:* See Acknowledgments on page 1816.

From the Worldwide Network of Blood and Marrow Transplantation. Presented in part at the BMT Tandem Meetings, Orlando, Florida, February 2017.

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<https://doi.org/10.1016/j.bbmt.2017.07.026>

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addressing the economic issues surrounding HCT, particularly in the developing world, which prompted considerable discussion (Do Stem Cell Transplants Need to be So Expensive?).

The complexity of these difficulties cannot be underappreciated or underestimated. The scientific, ethical, and economic challenges in delivering this intensive life-threatening treatment with wisdom, compassion, and cost-attentiveness is a stretch goal. Yet we also recognize that many patients who could benefit from HCT do not yet have access, in both the developed and developing parts of the world. Learning how to deliver transplantation care must include teaching ourselves and our successors to do it thoughtfully and cost-efficiently and will require new skills, new tools, and refocused attention.

Of course, these problems are of greater importance in those countries without the necessary economic resources to increase the number of patients who need this life-saving procedure, yet the lessons learned may be applied everywhere. Some published studies from Mexico and India describe strategies for performing HCT in developing countries, such as controlling daily hospital bed charges and physician fees, using more affordable generic drugs whenever possible, transitioning procedures to outpatient settings, avoiding cryopreservation of grafted cells, using reduced-intensity conditioning, using peripheral blood as a stem cell source, limiting transfusions, and avoiding expensive and repetitive diagnostic studies [4–9]. More information is needed on describing and testing proposed cost-efficient models that maximize financial resources without compromising patient safety. Such research will require collaboration among HCT centers across the developed and developing world.

Transplantation therapy can save many lives, but it must be delivered more broadly, and with truncated costs, to a larger population. If we do not address this expanding financial burden, then the necessary societal will, resources, and dollars will never be available to treat the many patients who we currently cannot serve. As a field, we should be proud and excited by the scope of improvements in the care that we deliver, but we have largely overlooked the dismal science of health economics. We should judge and measure the utility and value of each component of care delivered. This

represents a new area for disciplined investigation and a new opportunity for improvement—both are within the scope of science and compassionate care delivery. Compared with other therapies for these diseases, HCT generally has been shown to be a cost-effective therapeutic modality. Nevertheless, we need to continue to keep moving on the science, while also learning how to deliver better and more cost-efficient transplantation care.

## ACKNOWLEDGMENTS

*Financial disclosure:* The authors have nothing to disclose.

*Conflict of interest statement:* There are no conflicts of interest to report.

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