
Preface

The evolution of hematopoietic cell transplantation (HCT) as a science, discipline, and treatment modality for malignant diseases in children is integrally linked to the dramatic improvement in outcomes for children with cancer that have occurred during the past four to five decades. The biologic concepts leading to early clinical investigation of the transplantation of bone marrow from a normal donor to a compromised recipient began in treatment-refractory acute leukemia and congenital immunodeficiency. However, the number of potential clinical indications for HCT within just the spectrum of malignant diseases in children has expanded considerably beyond leukemia to a number of solid tumors, including those situations utilizing autologous HCT as rescue from high, both myeloablative and sub-myeloablative, doses of chemotherapy. In addition, the number of investigational and accepted clinical indications for HCT in nonmalignant diseases, including primary and secondary marrow failure disorders, hemoglobinopathies, congenital and acquired immunodeficiency states, and glycogen and lipid storage disorders, may now well outnumber the oncologic applications, but are beyond the scope of this text.

Advances in transplant techniques, improved rates of engraftment, expanded donor sources beyond matched siblings or family members, and the decrease in serious complications, notably acute and chronic graft versus host disease, paralleled advances in the understanding of the normal human immune system. Subsequently, therapeutic manipulation of host immune responses resulted in improved clinical outcomes with HCT. Similarly, mechanical and pharmacological manipulation of harvested hematopoietic stem cells through deletion or enhanced selection of specific cell populations has refined therapeutic efficacy by enhancing graft versus tumor effects and targeted tumor cell elimination.

Similar to the experience with chemotherapy and radiotherapy, where long-term, longitudinal follow-up of survivors has demonstrated significant acute and long-term toxicities, as HCT becomes increasingly successful in contributing to the survivor base, specific late effects are expected and observed. The potential for still unanticipated late effects of HCT warrants a focused survivorship research agenda.

The field of HCT for children with cancer is replete with extraordinary basic science and clinical investigators from numerous disciplines from all over the world. They are represented by legendary giants, including those who have been recognized for their efforts as Nobel laureates as well as by

the many who continue to work tirelessly managing critically ill patients. It is an incredible honor to have been mentored by and to have collaborated with such dedicated professionals who have contributed enormously to establishing HCT as a pivotal treatment modality in childhood cancer.

As with all scientific discovery that ultimately leads to clinical practice, the important role of clinical research in this translation cannot be overstated. Countless children and adolescents have served as willing participants in clinical trials. Many have benefitted personally from these experiences, and many more have contributed to the ever-expanding pool of knowledge that will directly benefit only those children who will follow. It is to those children and their families that we humbly and thankfully dedicate this book.

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