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ABSTRACT
The complexity of chronic graft-versus-host disease (GVHD) and the lack of established research methods have made it difficult to design, conduct, and analyze clinical trials involving subjects with this disease, even when promising treatment options are available. This consensus document was developed to offer an approach for overcoming these obstacles. Clinical trials in chronic GVHD should adhere to principles of good trial design and practice. Inclusion and exclusion criteria should allow as many subjects to participate as possible without compromising the interpretation of results. Pre-enrollment assessment of chronic GVHD characteristics should be standardized. The protocol should provide clear guidance about administration of study medication and other interventions. Methods of assessing response should be defined and validated in advance. Efficacy endpoints should be selected to reflect clinical benefit. Expert biostatistical support is needed to ensure the validity and reliability of trial results. The use of consistent standards in clinical trial designs to evaluate agents that have activity in pathogenic pathways could facilitate advances in the treatment of chronic GVHD.

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KEY WORDS
Chronic graft-versus-host disease ● Allogeneic hematopoietic cell transplantation ● Consensus ● Clinical trials ● Design

INTRODUCTION

Previous studies have provided a good understanding of risk factors for development of chronic graft-versus-host disease (GVHD) and risk factors for mortality among patients with newly diagnosed chronic GVHD. Advances in supportive care have decreased morbidity, but survival for patients with chronic GVHD has not changed since the mid-1980s. Five-year survival rates for patients with newly diagnosed “standard-risk”