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ABSTRACT

The lack of standardized criteria for quantitative measurement of therapeutic response in clinical trials poses a major obstacle for the development of new agents in chronic graft-versus-host disease (GVHD). This consensus document was developed to address several objectives for response criteria to be used in chronic GVHD-related clinical trials. The proposed measures should be practical for use both by transplantation and nontransplantation medical providers, adaptable for use in adults and in children, and focused on the most important chronic GVHD manifestations. The measures should also give preference to quantitative, rather than semiquantitative, measures; capture information regarding signs, symptoms, and function separately from each other; and use validated scales whenever possible to demonstrate improved patient outcomes and meet requirements for regulatory approval of novel agents. Based on these criteria, we propose a set of measures to be considered for use in clinical trials, and forms for data collection are provided (http://www.asbmt.org/GvHDForms). Measures should be made at 3-month intervals and whenever major changes are made in treatment. Provisional definitions of complete response, partial response, and progression are proposed for each organ and for overall outcomes. The proposed response criteria are based on current expert consensus.