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Abstract: Systemic mastocytosis (SM) is characterized by accumulation of neoplastic mast cells and is classified into indolent and aggressive forms. The latter include aggressive SM (ASM), mast cell leukemia (MCL), and SM associated with a myeloid neoplasm wherein 1 or both disease compartments exhibit advanced features. These variants, henceforth collectively referred to as advanced SM for the purposes of this report, are typically characterized by organ damage and shortened survival duration. In contrast to indolent SM, in which symptoms are usually managed by noncytotoxic antimediator therapy, cyto reduction is usually necessary for disease control in advanced SM. Unfortunately, current drug treatment of these patients rarely results in complete clinical and histopathologic remissions or improved survival time. Previously defined response criteria were adapted to the heterogeneous presentations of advanced SM and the limited effects of available drugs. However, recent advances in
understanding the molecular pathogenesis of SM and the corresponding prospect in targeted therapy make it a priority to modify these criteria. Our current study is the product of an international group of experts and summarizes the challenges in accomplishing this task and forwards a new proposal for response criteria, which builds on prior proposals and should facilitate response evaluation in clinical trials.