Steroid refractory chronic graft-versus-host disease (cGVHD) is associated with a significant morbidity and mortality. Although first-line treatment of cGVHD is based on controlled trials, second-line treatment is almost solely based on phase II trials or retrospective analyses. The consensus conference on clinical practice in cGVHD held in Regensburg aimed to achieve a consensus on the current evidence of treatment options as well as to provide guidelines for daily clinical practice. Treatment modalities are the use of steroids and calcineurin inhibitors as well as immunomodulating modalities (photopheresis, mTOR-inhibitors, thalidomide, hydroxychloroquine, vitamin A analogs, clofazimine), and cytostatic agents (mycophenolate mofetil, methotrexate, cyclophosphamide, pentostatin). Recent reports showed some efficacy of rituximab, alemtuzumab, and etanercept in selected patients. Moreover, tyrosine kinase inhibitors such as imatinib came into the field because of their ability to interfere with the platelet-derived growth factor (PDGF-R) pathway involved in fibrosis. An other treatment option is low-dose thoracoabdominal irradiation. Although different treatment options are available, the “trial-and-error system” remains the only way to identify the drug effective in the individual patient, and valid biomarkers are eagerly needed to identify the likelihood of response to a drug in advance. Moreover, the sparse evidence for most treatment entities indicates the urgent need for systematic evaluation of second-line treatment options in cGVHD.

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INTRODUCTION

Chronic graft-versus-host disease (cGVHD) remains the leading cause for late morbidity and mortality after allogeneic hematopoietic stem cell transplantation (HSCT). Although half of the patients respond to first-line treatment, prognosis of steroid refractory cGVHD remains poor [1-3]. Primary treatment of cGVHD is based on controlled trials and consists of prednisone given with or without a calcineurin inhibitor (CNI). In contrast, evidence in steroid refractory cGVHD is limited almost