About the Editor

Srdan Verstovsek

Srdan Verstovsek, Professor of Medicine in the Leukemia Department at The University of Texas MD Anderson Cancer Center, USA, is Director of the Clinical Research Center for Myeloproliferative Neoplasms (MPNs) and Chief of the Section for MPNs within the department. He maintains a very active clinic, with expertise in the treatment of MPNs, including Myelofibrosis, Polycythemia Vera, Essential Thrombocythemia, and others.

He has been a principal investigator of more than 40 clinical trials for patients with MPNs, including trials of all 10 JAK2 inhibitors tested to date. In addition, he was principal investigator of the COMFORT-I trial, which led to the approval of ruxolitinib as therapy for myelofibrosis. He is a regular speaker at national and international meetings and sits on the Medical Advisory Boards of several MPN patient societies. Dr. Verstovsek also maintains an active laboratory focused on testing and developing new therapies for MPN, as well as performing translational research using patient samples to evaluate clinical trial performance and further understand the pathophysiology of MPNs.

Foreword

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Myelofibrosis (MF) is characterized by proliferation of a myeloid-derived stem cell clone, which manifests as megakaryocyte proliferation with atypia, extramedullary hematopoiesis, bone marrow fibrosis, osteosclerosis and leukoerythroblastosis [1]. Disease-related symptoms include fatigue, night sweats, fever, weight loss, bone pain and others. Main causes of death include bone marrow failure, transformation to leukemia, thrombosis, bleeding, infections and portal hypertension [2]. The majority of patients are diagnosed with MF after aged 60 years and median survival ranges from 2 to 11 years, depending of the presence of several risk factors [3,4]. Recent estimates of the incidence rates of MF in the United States and Europe range from approximately 0.5 to 1/100,000 per year [5,6]. However, given the aging population, the incidence is expected to increase in coming years.

Although MF was first described in 1879 [7] and further classified as a myeloproliferative disorder in 1951 [8], treatment of the disease has focused mainly on palliation. However, discovery of the acquired *JAK2* V617F mutation in up to 60% of patients with MF in 2005 [9] ushered in a new era, which has led to the development of novel therapies for MF (*JAK2* inhibitors) and renewed interest in unraveling the complex pathobiology of the disease. In 2011, ruxolitinib was the first and to date only therapy approved by the US FDA for the treatment of MF, and the European Medicines Agency followed suit in 2012. Ruxolitinib has been shown to significantly reduce splenomegaly and constitutional symptoms, improve quality of life and prolong survival [10]. In addition, renewed interest in the field has led to more

clinical trials with new therapies and a search for other drivers of disease pathogenesis [11].

Recent advances in treatment and our understanding of the pathophysiology of MF are highlighted in this book. The most recent guidelines for diagnosis, risk stratification and prognostication are reviewed, highlighting the complexity encountered in the field of MF. Once a patient is diagnosed, making prognostic predictions based on clinical manifestations and symptoms are a key for developing the proper treatment plan. Recent advances in understanding disease burden and delineating the goals of treatment are also reviewed. In addition, the role of and therapy for extramedullary hematopoiesis and splenomegaly in MF is reviewed. The latter chapters review the most recent clinical data on current treatments for MF, along with the management of symptoms and drug toxicities. Despite progress in the treatment of MF, to date the only curative treatment is hematopoietic stem cell transplantation, a procedure for which fewer than 10% of patients are eligible owing to age and comorbidities. The final chapter reviews the most recent criteria for selecting MF patients for hematopoietic cell transplant.

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