



Advancing the Myelofibrosis Treatment Paradigm: A Case-based Collaborative for the Advanced Practitioner in Oncology

Arber DA, Orazi A, Hasserjian R, et al. The 2016 revision to the World Health Organization classification of myeloid neoplasms and acute leukemia. *Blood*. 2016;127(20):2391-2405.

Barbui T, Thiele J, Gisslinger H, et al. The 2016 revision of WHO classification of myeloproliferative neoplasms: clinical and molecular advances. *Blood Rev*. 2016;30(6):453-459.

Barosi G, Mesa RA, Thiele J, et al; International Working Group for Myelofibrosis Research and Treatment (IWG-MRT). Proposed criteria for the diagnosis of post-polycythemia vera and post-essential thrombocythemia myelofibrosis: a consensus statement from the International Working Group for Myelofibrosis Research and Treatment. *Leukemia*. 2008;22(2):437-438.

Cerquozzi S, Tefferi A. Blast transformation and fibrotic progression in polycythemia vera and essential thrombocythemia: a literature review of incidence and risk factors. *Blood Cancer J*. 2015;5(11):e366.

Cervantes F, Ross DM, Radinoff A, et al. Efficacy and safety of a novel dosing strategy for ruxolitinib in the treatment of patients with myelofibrosis and anemia: the REALISE phase 2 study. *Leukemia*. 2021;35(12):3455-3465.

Chifotides HT, Bose P, Verstovsek S. Momelotinib: an emerging treatment for myelofibrosis patients with anemia. *J Hematol Oncol*. 2022;15(1):7.

Chifotides HT, Verstovsek S, Bose P. Association of myelofibrosis phenotypes with clinical manifestations, molecular profiles, and treatments. *Cancers*. 2023;15(13):3331.

Coltro G, Mannelli F, Loscocco GG, et al. Differential prognostic impact of cytopenic phenotype in prefibrotic vs overt primary myelofibrosis. *Blood Cancer J*. 2022;12(8):116.

Drugs@FDA: FDA-Approved Drugs. Fedratinib. May 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/212327s005lbl.pdf. Accessed November 2023.

Drugs@FDA: FDA-Approved Drugs. Momelotinib. September 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/216873s000lbl.pdf. Accessed November 2023.

Drugs@FDA: FDA-Approved Drugs. Pacritinib. August 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/208712s001lbl.pdf. Accessed November 2023.



Advancing the Myelofibrosis Treatment Paradigm: A Case-based Collaborative for the Advanced Practitioner in Oncology

Drugs@FDA: FDA-Approved Drugs. Ruxolitinib. January 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/202192s028lbl.pdf. Accessed November 2023.

Fisher DAC, Miner CA, Engle EK, et al. Cytokine production in myelofibrosis exhibits differential responsiveness to JAK-STAT, MAP kinase, and NFκB signaling. *Leukemia*. 2019;33(8):1978-1995.

Gangat N, Kuykendall A, Al Ali N, et al. Black African-American patients with primary myelofibrosis: a comparative analysis of phenotype and survival. *Blood Adv*. 2023;7(12):2694-2698.

Gangat N, Caramazza D, Vaidya R, et al. DIPSS plus: a refined Dynamic International Prognostic Scoring System for primary myelofibrosis that incorporates prognostic information from karyotype, platelet count, and transfusion status. *J Clin Oncol*. 2011;29(4):392-397.

Garmezay B, Schaefer JK, Mercer J, et al. A provider's guide to primary myelofibrosis: pathophysiology, diagnosis, and management. *Blood Rev*. 2021;45:100691.

Hammami MB, Yang J, Thakur R, et al. Examining racial disparities in the incidence and survival of myelofibrosis: insights from SEER database and an institutional cohort (2000-2020). Presented at: 2023 Society of Hematologic Oncology (SOHO). Annual Meeting; September 6-9, 2023; Houston, TX. Abstract MPN-470.

Harrison CN, Garcia JS, Somerville TCP, et al. Addition of navitoclax to ongoing ruxolitinib therapy for patients with myelofibrosis with progression or suboptimal response: phase II safety and efficacy. *J Clin Oncol*. 2022;40(15):1671-1680.

Harrison CN, Vannucchi AM, Platzbecker U, et al. Momelotinib versus best available therapy in patients with myelofibrosis previously treated with ruxolitinib (SIMPLIFY 2): a randomised, open-label, phase 3 trial. *Lancet Haematol*. 2018;5(2):e73-e81.

Ibrahim U, Petrone GEM, Mascarenhas J, et al. Peritransplantation use of ruxolitinib in myelofibrosis. *Biol Blood Marrow Transplant*. 2020;26(12):2177-2180.

Langlais BT, Geyer H, Scherber R, et al. Quality of life and symptom burden among myeloproliferative neoplasm patients: do symptoms impact quality of life? *Leuk Lymphoma*. 2019;60(2):402-408.

Leiva O, Hobbs G, Ravid K, et al. Cardiovascular disease in myeloproliferative neoplasms: JACC: CardioOncology State-of-the-Art Review. *JACC CardioOncol*. 2022;4(2):166-182.



Advancing the Myelofibrosis Treatment Paradigm: A Case-based Collaborative for the Advanced Practitioner in Oncology

Masarova L, Alhuraiji A, Bose P, et al. Significance of thrombocytopenia in patients with primary and postessential thrombocythemia/polycythemia vera myelofibrosis. *Eur J Haematol*. 2018;100(3):257-263.

Mascarenhas J, Komrokji RS, Palandri F, et al. Randomized, single-blind, multicenter phase II study of two doses of imetelstat in relapsed or refractory myelofibrosis. *J Clin Oncol*. 2021;39(26):2881-2892.

Mascarenhas J, Kremyanskaya M, Patriarca A, et al. MANIFEST: pelabresib in combination with ruxolitinib for janus kinase inhibitor treatment-naïve myelofibrosis. *J Clin Oncol*. 2023:JC02201972.

Mascarenhas J, Gleitz HFE, Chifotides HT, et al. Biological drivers of clinical phenotype in myelofibrosis. *Leukemia*. 2023;37(2):255-264.

Mesa RA, Kiladjian JJ, Catalano JV, et al. SIMPLIFY-1: a phase III randomized trial of momelotinib versus ruxolitinib in janus kinase inhibitor-naïve patients with myelofibrosis. *J Clin Oncol*. 2017;35(34):3844-3850.

Morris R, Kershaw NJ, Babon JJ. The molecular details of cytokine signaling via the JAK/STAT pathway. *Protein Sci*. 2018;27(12):1984-2009.

Morsia E, Torre E, Poloni A, et al. Molecular pathogenesis of myeloproliferative neoplasms: from molecular landscape to therapeutic implications. *Int J Mol Sci*. 2022;23(9):4573.

National Comprehensive Cancer Network. Myeloproliferative Neoplasms (Version 2.2023). Updated October 25, 2023. https://www.nccn.org/professionals/physician_gls/pdf/mpn.pdf. Accessed November 2023.

National Comprehensive Cancer Network. Prevention and Treatment of Cancer-Related Infections (Version 1.2023). Updated June 28, 2023. https://www.nccn.org/professionals/physician_gls/pdf/infections.pdf. Accessed November 2023.

Oh ST, Talpaz M, Gerds AT, et al. ACVR1/JAK1/JAK2 inhibitor momelotinib reverses transfusion dependency and suppresses hepcidin in myelofibrosis phase 2 trial. *Blood Adv*. 2020;4(18):4282-4291.

O'Sullivan JM, Harrison CN. Myelofibrosis: clinicopathologic features, prognosis, and management. *Clin Adv Hematol Oncol*. 2018;16(2):121-131.



Advancing the Myelofibrosis Treatment Paradigm: A Case-based Collaborative for the Advanced Practitioner in Oncology

Padrnos L, Scherber R, Geyer H, et al. Depressive symptoms and myeloproliferative neoplasms: Understanding the confounding factor in a complex condition. *Cancer Med*. 2020;9(22):8301-8309.

Palandri F, Palumbo GA, Elli EM, et al. Ruxolitinib discontinuation syndrome: incidence, risk factors, and management in 251 patients with myelofibrosis. *Blood Cancer J*. 2021;11(1):4.

Pemmaraju N, Verstovsek S, Mesa R, et al. Defining disease modification in myelofibrosis in the era of targeted therapy. *Cancer*. 2022;128(13):2420-2432.

Potluri J, Harb J, Masud AA, et al. A phase 3, double-blind, placebo-controlled, randomized study evaluating navitoclax in combination with ruxolitinib in patients with myelofibrosis (TRANSFORM-1). *Blood*. 2020;136(Supplement 1):4.

Primary myelofibrosis. Orphanet. Updated May 2019. Accessed November 2023. https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Ing=EN&Expert=824.

Reis E, Buonpane R, Celik H, et al. Discovery of INCA033989, a monoclonal antibody that selectively antagonizes mutant calreticulin oncogenic function in myeloproliferative neoplasms (MPNs). *Blood*. 2022;140(Supplement 1):14-15.

Reynolds SB, Pettit K. New approaches to tackle cytopenic myelofibrosis. *Hematology Am Soc Hematol Educ Program*. 2022;2022(1):235-244.

Rumi E, Trotti C, Vanni D, et al. The genetic basis of primary myelofibrosis and its clinical relevance. *Int J Mol Sci*. 2020;21(23):8885.

Rumi E, Pietra D, Pascutto C, et al; Associazione Italiana per la Ricerca sul Cancro Gruppo Italiano Malattie Mieloproliferative Investigators. Clinical effect of driver mutations of JAK2, CALR, or MPL in primary myelofibrosis. *Blood*. 2014;124(7):1062-1069.

Shammo JM, Stein BL. Mutations in MPNs: prognostic implications, window to biology, and impact on treatment decisions. *Hematology Am Soc Hematol Educ Program*. 2016;2016(1):552-560.

Singer JW, Fleischman A, Al-Fayoumi S, et al. Inhibition of interleukin-1 receptor-associated kinase 1 (IRAK1) as a therapeutic strategy. *Oncotarget*. 2018;9(70):33416-33439.

Tefferi A. Primary myelofibrosis. National Organization for Rare Disorders. Updated June 24, 2020. Accessed November 2023. <https://rarediseases.org/rare-diseases/primary-myelofibrosis/>.



Bibliography and Suggested Reading

Advancing the Myelofibrosis Treatment Paradigm: A Case-based Collaborative for the Advanced Practitioner in Oncology

- Tefferi A. Primary myelofibrosis: 2023 update on diagnosis, risk-stratification, and management. *Am J Hematol.* 2023;98(5):801-821.
- Tefferi A, Guglielmelli P, Nicolosi M, et al. GIPSS: genetically inspired prognostic scoring system for primary myelofibrosis. *Leukemia.* 2018;32(7):1631-1642.
- Tefferi A. Primary myelofibrosis: 2021 update on diagnosis, risk-stratification and management. *Am J Hematol.* 2021;96(1):145-162.
- Tremblay D, Mesa R. Addressing symptom burden in myeloproliferative neoplasms. *Best Pract Res Clin Haematol.* 2022;35(2):101372.
- U.S. National Library of Medicine. An efficacy and safety study of luspatercept (ACE-536) versus placebo in subjects with myeloproliferative neoplasm-associated myelofibrosis on concomitant JAK2 inhibitor therapy and who require red blood cell transfusions (INDEPENDENCE). ClinicalTrials.gov identifier: NCT04717414. Updated August 14, 2023. Accessed November 2023. <https://clinicaltrials.gov/study/NCT04717414>.
- Verstovsek S, Gerds AT, Vannucchi AM, et al; MOMENTUM Study Investigators. Momelotinib versus danazol in symptomatic patients with anaemia and myelofibrosis (MOMENTUM): results from an international, double-blind, randomised, controlled, phase 3 study. *Lancet.* 2023;401(10373):269-280.
- Verstovsek S, Mesa RA, Sullivan S, et al. Advancing personalized care for patients with myeloproliferative neoplasms (MPNs): findings from a phase 2 scale-up quality initiative across two large U.S. oncology systems. *Blood.* 2021;138(Supplement 1):1911.
- Zhou A, Oh ST. Prognostication in MF: from CBC to cytogenetics to molecular markers. *Best Pract Res Clin Haematol.* 2014;27(2):155-164.