

New Frontiers in the Targeted Treatment of Myelofibrosis

References (Order of Appearance)

Chifotides HT, Bose P, Verstovsek S. Momelotinib: an emerging treatment for myelofibrosis patients with anemia. *J Hematol Oncol*. 2022;15(1):7. doi:[10.1186/s13045-021-01157-4](https://doi.org/10.1186/s13045-021-01157-4)

Fenaux P, Kiladjian JJ, Platzbecker U. Luspatercept for the treatment of anemia in myelodysplastic syndromes and primary myelofibrosis. *Blood*. 2019;133(8):790-794. doi:[10.1182/blood-2018-11-876888](https://doi.org/10.1182/blood-2018-11-876888)

Tremblay D, Mascarenhas J. Next Generation Therapeutics for the Treatment of Myelofibrosis. *Cells*. 2021;10(5):1034. doi:[10.3390/cells10051034](https://doi.org/10.3390/cells10051034)

Mascarenhas J, Hoffman R, Talpaz M, et al. Pacritinib vs Best Available Therapy, Including Ruxolitinib, in Patients With Myelofibrosis: A Randomized Clinical Trial. *JAMA Oncol*. 2018;4(5):652-659. doi:[10.1001/jamaoncol.2017.5818](https://doi.org/10.1001/jamaoncol.2017.5818)

Mesa RA, Vannucchi AM, Mead A, et al. Pacritinib versus best available therapy for the treatment of myelofibrosis irrespective of baseline cytopenias (PERSIST-1): an international, randomised, phase 3 trial. *Lancet Haematol*. 2017;4(5):e225-e236. doi:[10.1016/S2352-3026\(17\)30027-3](https://doi.org/10.1016/S2352-3026(17)30027-3)

Verstovsek S, Mesa R, Talpaz M, et al. Retrospective analysis of pacritinib in patients with myelofibrosis and severe thrombocytopenia. *Haematologica*. 2022;107(7):1599-1607. doi:[10.3324/haematol.2021.279415](https://doi.org/10.3324/haematol.2021.279415)

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. doi:[10.1016/S2352-3026\(17\)30237-5](https://doi.org/10.1016/S2352-3026(17)30237-5)

Mesa RA, Kiladjian JJ, Catalano JV, et al. SIMPLIFY-1: A Phase III Randomized Trial of Momelotinib Versus Ruxolitinib in Janus Kinase Inhibitor-Naive Patients With Myelofibrosis. *J Clin Oncol*. 2017;35(34):3844-3850. doi:[10.1200/JCO.2017.73.4418](https://doi.org/10.1200/JCO.2017.73.4418)

Mesa R, Harrison C, Oh ST, et al. Overall survival in the SIMPLIFY-1 and SIMPLIFY-2 phase 3 trials of momelotinib in patients with myelofibrosis. *Leukemia*. 2022;36(9):2261-2268. doi:[10.1038/s41375-022-01637-7](https://doi.org/10.1038/s41375-022-01637-7)

Verstovsek S, Gerds AT, Vannucchi AM, et al. 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. doi:[10.1016/S0140-6736\(22\)02036-0](https://doi.org/10.1016/S0140-6736(22)02036-0)

Gerds AT, Vannucchi AM, Passamonti F, et al. A Phase 2 Study of Luspatercept in Patients with Myelofibrosis-Associated Anemia. *Blood*. 2019;134(Supplement_1):557. doi:[10.1182/blood-2019-122546](https://doi.org/10.1182/blood-2019-122546)

New Frontiers in the Targeted Treatment of Myelofibrosis

References (Order of Appearance)

Gerds AT, Vannucchi AM, Passamonti F, et al. Duration of Response to Luspatercept in Patients (Pts) Requiring Red Blood Cell (RBC) Transfusions with Myelofibrosis (MF) - Updated Data from the Phase 2 ACE-536-MF-001 Study. *Blood*. 2020;136(Supplement 1):47-48. doi:[10.1182/blood-2020-137265](https://doi.org/10.1182/blood-2020-137265)

Yacoub A, Borate U, Rampal RK, et al. Efficacy and Safety of Add-on Parsaclisib to Ruxolitinib Therapy in Myelofibrosis Patients With Suboptimal Response to Ruxolitinib: Final Results From a Phase 2 Study. *Blood*. 2022;140(Supplement 1):579-582. doi:[10.1182/blood-2022-160384](https://doi.org/10.1182/blood-2022-160384)

Mascarenhas J, Kremianskaya M, Patriarca A, et al. MANIFEST: Pelabresib in Combination With Ruxolitinib for Janus Kinase Inhibitor Treatment-Naive Myelofibrosis. *J Clin Oncol*. 2023;JCO2201972. doi:[10.1200/JCO.22.01972](https://doi.org/10.1200/JCO.22.01972)

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. doi:[10.1200/JCO.21.02188](https://doi.org/10.1200/JCO.21.02188)

Vachani P, Lange A, Delgado RG, et al. Potential Disease-Modifying Activity of Navtemadlin (KRT-232), a First-in-Class MDM2 Inhibitor, Correlates with Clinical Benefits in Relapsed/Refractory Myelofibrosis (MF). *Blood*. 2021;138(Supplement 1):3581. doi:[10.1182/blood-2021-147543](https://doi.org/10.1182/blood-2021-147543)

Verstovsek S, Al-Ali HK, Mascarenhas J, et al. BOREAS: a global, phase III study of the MDM2 inhibitor navtemadlin (KRT-232) in relapsed/refractory myelofibrosis. *Future Oncol*. 2022;18(37):4059-4069. doi:[10.2217/fon-2022-0901](https://doi.org/10.2217/fon-2022-0901)

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. doi:[10.1200/JCO.20.02864](https://doi.org/10.1200/JCO.20.02864)