Feasibility Assessment of Myelofibrosis in Europe (Germany and France)

Study Description	
Background	Myelofibrosis (MF) is a rare, chronic myeloproliferative neoplasm characterized by progressive bone marrow fibrosis, extramedullary hematopoiesis, and marked splenomegaly. It may arise as primary MF (PMF) or develop secondary to polycythemia vera (post-PV MF) or essential thrombocythemia (post-ET MF).[1,2] MF predominantly affects older patients, with a median age at diagnosis of approximately 65 years for PMF. Annual incidence is approximately 0.5 to 1.5 per 100,000 inhabitants, with Germany reporting the highest prevalence among EU countries at 1.2 to 1.8 per 100,000.[3,4] Approximately 90% of patients carry a driver mutation in JAK2, CALR, or MPL, leading to constitutive activation of the JAK-STAT signaling pathway.[5] JAK inhibitors (JAKi) are now an indispensable component of pharmacological MF therapy. Ruxolitinib (JAK1/2 inhibitor) represents the established first-line standard of care, and additional JAKi are now available for previously treated patients and specific subgroups. Allogeneic stem cell transplantation, however, remains the only potentially curative approach.[6,7] Nevertheless, robust real-world data on the hematological care of MF patients in Germany and France remain lacking. The present feasibility study aims to lay the groundwork for a structured TherapyMonitor, with a focus on center representativeness, data availability, and the quality of care-relevant parameters in routine clinical practice. **References** [1] Passamonti F, Mora B. Myelofibrosis. Blood. 2023;141(16):1954–1970. doi:10.1182/blood.2022017423 [2] Tefferi A. Primary myelofibrosis: 2023 update on diagnosis, risk-stratification, and management. Am J Hematol. 2023;98(5):801–821. doi:10.1002/ajh.26857 [3] Slowley A, et al. Myelofibrosis and anemia: A German claims data analysis to describe epidemiology and current treatment. Eur J Haematol. 2024. doi:10.1111/ejh.14284 [4] Merker L, Plundrich D, Müller N, et al. Epidemiology and institutional treatment distribution of patients with myelofibrosis in Germany: Insights from a real-world evidence study. ISPOR Europe 2025; Glasgow, Scotland. [5] Leong DP, et al. Biological drivers of clinical phenotype in myelofibrosis. Leukemia. 2023;37:255–264. doi:10.1038/s41375-022-01767-y [6] Harrison CN, et al. JAK inhibitor selection in challenging scenarios of myelofibrosis: a review. Haematologica. 2025. doi:10.3324/haematol.2024.286969 [7] Mughal TI, et al. Management of splenomegaly in patients with myelofibrosis in the era of JAK inhibitors: a comprehensive review. Front Oncol. 2026. doi:10.3389/fonc.2026.1851781
Objectives	The objectives are: • To conduct a **preliminary assessment** to determine the feasibility of a potential study with a TherapyMonitor, with a particular focus on the availability and quality of relevant data sources.

	• To collect **aggregated data** on the epidemiology and care structure of the target populations from primary sources, defined as relevant institutions treating MF in the selected countries (Germany and France), to enable a representative sample of affected individuals and institutions. • To **identify and contractually engage data sources,** defined as relevant institutions treating MF, particularly within the target subpopulation, in order to assess the feasibility of chart reviews.
Methodology	In this project phase, a comprehensive analysis of oncological care structures in the selected countries (France and Germany) will be conducted to develop a representative sampling framework. This includes the identification of institutions involved in the treatment of patients with MF. In addition, the feasibility assessment is intended to enable a better understanding of the following aspects: • Patients with a PMF or SMF diagnosis • Standard of care and treatment pathways • Access to relevant patient data • Interest and capacity of centers to participate • Estimated total sample size and country-specific recruitment targets This process will result in a stratified list of oncology centers across four key care sectors: • University hospitals • Non-university hospitals • Outpatient specialty centers (MVZ) • Office-based oncologists (private practice) This stratification forms the basis for a representative sample and enables the extrapolation of prevalence and incidence data within the target population.
Data Collection Period	The start of the data collection phase is planned for **September 2026.**
Study Period	Aggregated data from MF cases will be collected for the period from **1st of January 2025** to **31st of December 2025**.