Abstract: P2029

Title: THE CLINICAL BURDEN OF MYELOFIBROSIS AND ASSOCIATED ANEMIA IN FRANCE

Abstract Type: e-Poster Presentation

Topic: Myeloproliferative neoplasms - Clinical

Background:

Myelofibrosis (MF) is driven by genetic mutations in bone marrow stem cells; Janus kinase 2, calreticulin, and myeloproliferative leukemia virus mutations are most common. Treatments aim to alleviate symptoms, reduce spleen size, improve blood cell counts, and mitigate complications. Recently developed JAK inhibitors (JAKi) have shown promise in improving MF outcomes, but they can exacerbate anemia. The proportion of MF patients with anemia increases with the course of disease, and the need for red blood cell transfusions has been described as an independent adverse risk factor for both overall survival and leukemic transformation.

Aims:

There is a lack of real-world evidence on the clinical burden of MF in France. This study describes treatment patterns, and clinical burden of disease and anemia in patients with MF in France to document the unmet medical need in these populations, and to contextualize the potential impact of new treatments.

Methods:

This was a retrospective observational study of administrative healthcare claims (secondary data) from the national '*Système National des Données de Santé'* (SNDS) database from January 1, 2011, to December 31, 2022. Algorithms and International Classification of Diseases codes from hospitalization diagnosis to identify patients with MF and anemia were explored and validated based on clinical experts' feedback, to extract relevant patients' information. The baseline period was 24 months prior to the MF index date. There was a minimum of 12 months follow-up after MF diagnosis.

Results:

Overall, 3667 patients with MF were identified in the SNDS between January 1, 2013, and December 31, 2021. 35% had secondary MF, and 49% had evidence of anemia (i.e. anemia diagnosis, reimbursed drug prescription records, and/or transfusion at baseline). The median age of patients was 71 years, and 57% were males. Average follow-up was 3.3 years since MF diagnosis.

During follow-up, a specific MF treatment was given to 2535 (69%) MF patients; their treatment pathways are summarized in **Figure 1 (Sankey diagram)**. Systemic glucocorticoids (52%), ruxolitinib (51%), and hydroxyurea (32%) were the most frequent MF treatments received. A total of 59% of patients received at least 1 blood transfusion during follow-up and 45% received erythropoiesis-stimulating agents. Infections (52%) were the most frequent complications reported; patients also commonly experienced thrombocytopenia (28%), major bleeding (28%), and splenomegaly (15%).

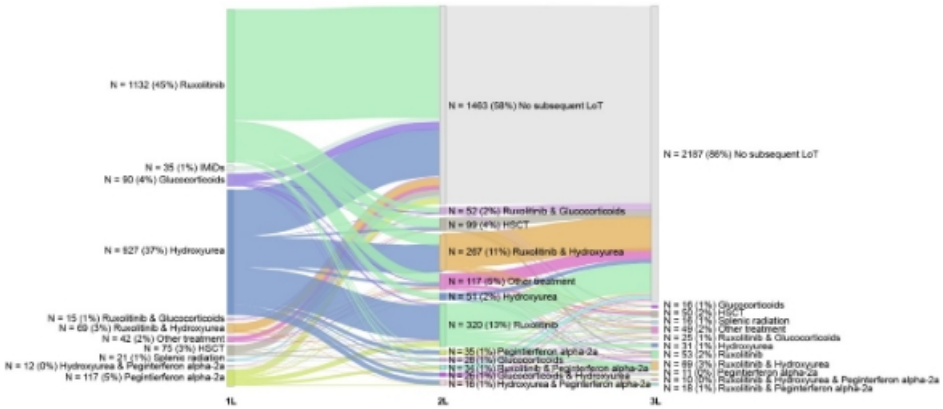
Overall, 12% of patients progressed to acute myeloid leukemia, 38% developed transfusion dependence, and 56% died. Median survival was 4.1 years from MF diagnosis.

Summary/Conclusion:

This analysis of real-world data identified a nationwide, large cohort** of French** patients with MF. Despite limitations associated with the identification of MF via hospitalization diagnosis codes, the number and profile of patients seem consistent with the previously described epidemiology in France. The clinical burden of MF and associated anemia remains high in France. JAKi and potential future MF treatments that address both symptom relief and anemia are needed.** Further analyses in patient subgroups of interest are underway to

better understand the unmet medical need.

Figure 1: Treatment Regimens for MF (Sankey Diagram)



1L, first line; 2L, second line; 3L, third line; LoT, line of treatment.

Keywords: Anemia, Myelofibrosis