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Epidemiology and Institutional Treatment Distribution of Patients with Myelofibrosis in Germany

Insights from a Real-World Evidence Study

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INTRODUCTION

Myelofibrosis (MF)
is a rare chronic myeloid malignancy characterized by the development of **fibrotic scar tissue** in the bone marrow, which disrupts normal hematopoiesis and results in **anemia, splenomegaly**, and a spectrum of systemic symptoms[1].
MF may occur de novo due to somatic mutations in genes such as *JAK2*, *CALR*, or *MPL*, or it can arise secondary to the progression of **polycythemia vera** or **essential thrombocythemia**[1]. Disease course is highly variable, with some patients remaining asymptomatic for years, while others experience rapid progression or transformation to **acute myeloid leukemia (AML)**[2].

Treatment Strategies
Are guided by disease severity and symptom burden. **Janus kinase inhibitors (JAKi)** are frequently used to alleviate symptoms and reduce spleen volume[3].

Real-World Data
on MF epidemiology and treatment patterns remain limited, particularly in Germany. This study aimed to:

- Estimate the **incidence and prevalence** of MF in Germany in 2022.
- Evaluate **treatment patterns** across different helthcare settings in a **representative patient cohort**

METHODS

A Health Care Structure Analysis (HCSA)
identified relevant MF-treating centers using the **2020 hospital quality reports** published by the German Federal Joint Committee (**G-BA**).
Across Germany, **346 MF-treating centers** are care relevant, including:

- Office-Based Practices / Medical Care Centers (OBP/MCC)
- Non-University Hospitals (NUH)
- University Hospitals (UH)

An Epidemiologic Survey
was distributed to MF-treating institutions to collect data on **diagnosed and treated patients** during the first half of 2022 (**HY1 2022**).

- **45 institutions** participated, reporting prevalence and incidence data for **818 MF patients**.
- Institutional weighting was applied to project national estimates for **total prevalence** (including patients under watch-and-wait (WAW)), **treated prevalence** and **incidence**.

A Retrospective Chart Review
of a **representative sample** of MF-treating institutions contributed detailed patient-level data.

- **54 institutions** provided data on **350 MF patients** treated in 2021.
- The review captured the **entire treatment history** from initial diagnosis to current therapy status, enabling analysis of care pathways and institutional treatment patterns.

RESULTS

Epidemiologic Survey

The **346 MF-treating** centers identified in the HCSA formed the basis for institutional weighting, to project prevalence and incidence from **818 patients** reported in the survey.

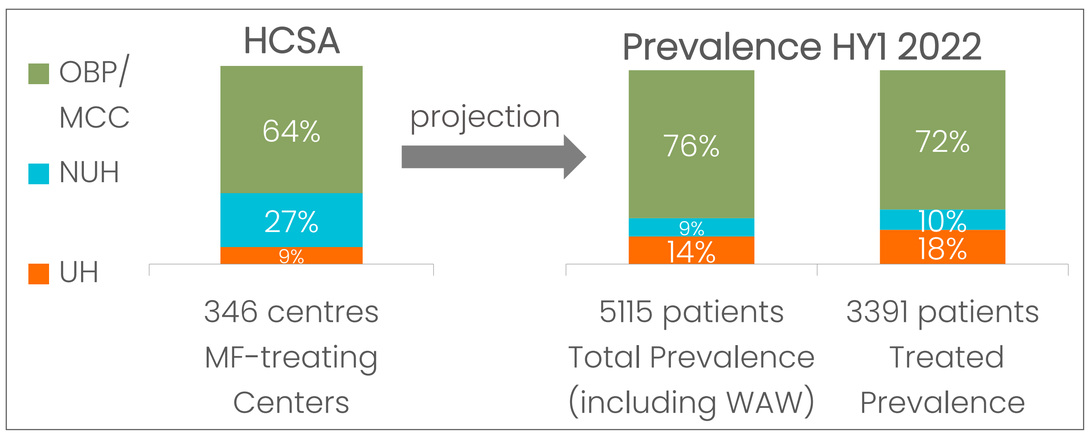

Figure 1. Institutional distribution of HY1 2022 prevalence.

Incident cases accounted for **26%** of total prevalence.
Full-year 2022 lower and upper estimates:

- **Prevalence**: 4273–6445 patients
- **Incidence**: 1111–1676 patients

Retrospective Chart Review

The institutional distribution of the chart review sample closely mirrors that of the epidemiologic survey, supporting its **representativeness**.

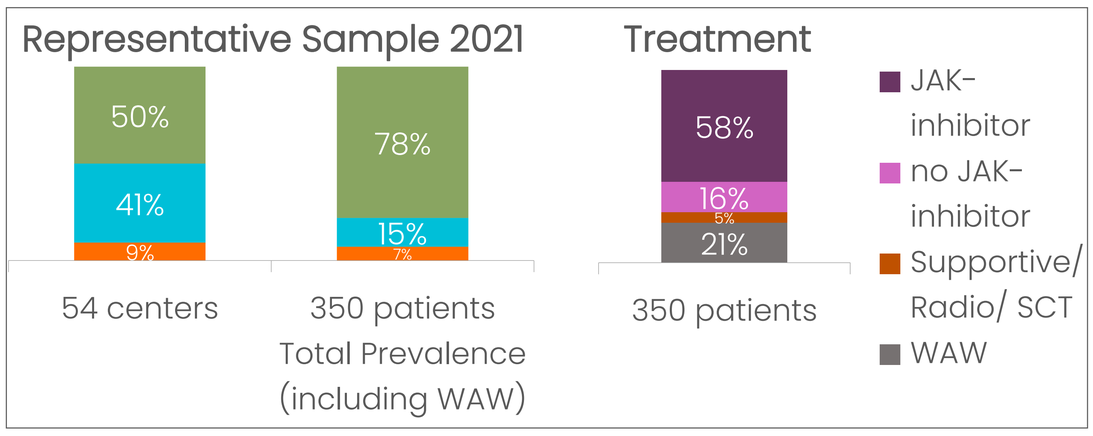

Figure 2. Representative sample and treatment patterns.

Over half of the patients received a **JAKi**, primarily **ruxolitinib**.
Among those not treated with a JAKi, **hydroxyurea** was the most common alternative. Patients with low-risk disease and minimal symptoms are typically managed with a WAW approach.

CONCLUSION

This real-world study provides updated insights into the **epidemiology and treatment patterns** of MF in Germany.

- Estimated **prevalence and incidence** fall within the **upper range of previously reported data**[4] reflecting the strength and representativeness of the institutional sample and methodology.
- Patient care is predominantly delivered by **OBPs** and **MCCs** highlighting the central role of outpatient hematology in disease management in Germany.
- **JAKi** are the **standard of care** for patients not managed with WAW, as demonstrated in the representative chart review sample.

The findings offer a valuable reference point for understanding MF care delivery and treatment choices in routine practice.

LIMITATIONS

Institutional Weighting relied on consistent reporting behavior across institution types, and could be biased due to **voluntary participation**.
While robust data quality processes were applied to minimize manual entry errors, inconsistencies originating from **variability in clinical documentation** and **record-keeping practices** could not be controlled.

References
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