Experienced or Perceived Burdens and Associated Quality-of-Life Impacts of Anemia and Transfusion Dependence in Myelofibrosis: A Patient Self-Report Survey Analysis

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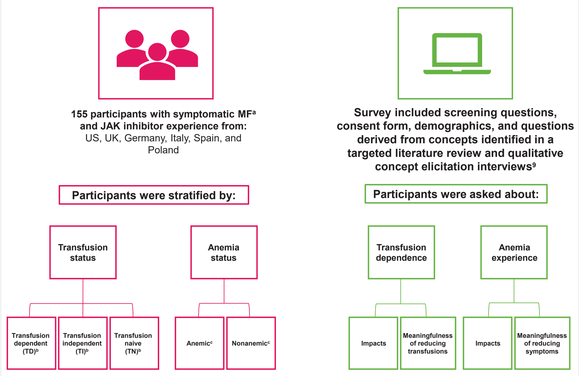
Background

- Myelofibrosis (MF) is associated with several debilitating symptoms, which often negatively impact patients' health-related quality of life (QOL) and functioning[1-3]
- While Janus kinase (JAK) inhibitors are the standard of care in MF, some can exacerbate anemia, a hallmark feature of MF that often increases in severity with disease progression[4]
- Anemia management in MF often requires red blood cell transfusions, which are an independent predictor of poor survival, are inversely correlated with QOL, and may lead to iron overload, which increases the risk of infections[5-8]
- Previously, qualitative concept elicitation interviews demonstrated substantial negative experiences with and perceptions of anemia and transfusions in 20 participants with MF who were either transfusion dependent (TD) or transfusion independent (TI)[9]
  - Here we expand on these findings by quantitatively evaluating the reported burden and associated impact of transfusion dependence on QOL and highlighting the importance of avoiding transfusion dependence in a larger sample of participants with MF

Study Design

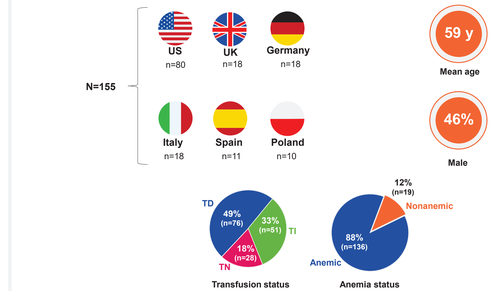
Figure 1: **Study Design**



JAK, Janus kinase; MF, myelofibrosis; MFSAF, Myelofibrosis Symptom Assessment Form; TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive. [a] Symptomatic MF is defined as a Total Symptom Score of ≥10 on the MFSAF version 4.0. [b] TD was defined as ≥2 transfusions (if MF diagnosed ≤3 months prior) or ≥1 transfusion every 3 months since diagnosis (if MF diagnosed >3 months prior), TN as never receiving a transfusion, and TI as not meeting criteria for TD or TN. [c] Anemia was defined by TD status or if participants self-reported that their healthcare provider had diagnosed them as anemic.

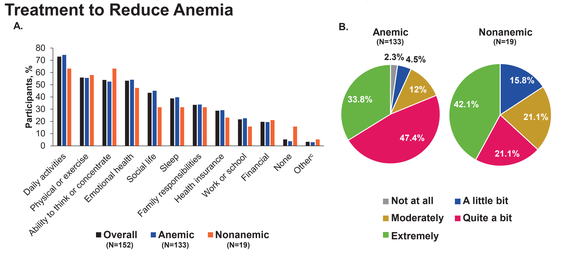
Results

Figure 2: **Baseline Characteristics**



TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive.

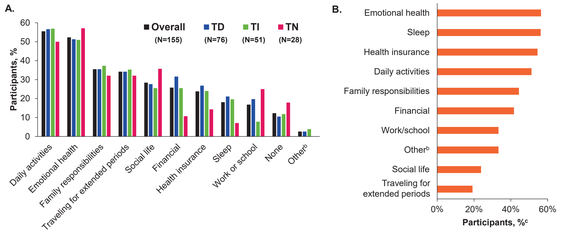
Figure 3: **Reported (A) Anemia Impacts[a,b] and (B) Importance of Treatment to Reduce Anemia**



Most participants with or without an anemia diagnosis experienced anemia-related symptoms and reported that improving anemia as a treatment outcome was "extremely" or "quite a bit" important

[a] Responses are shown in order of descending frequency from left to right. [b] Question was posed as experienced impacts for those who were anemic and anticipated impacts for those who were nonanemic. [c] Other includes lack of appetite, weak, tired, all symptoms mentioned, lack of oxygen, risk of falls, enjoyment of life, and not getting enough rest.

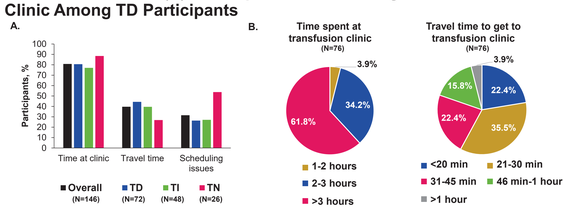
Figure 4: **Reported (A) Impacts of Frequent Blood Transfusions[a] and (B) Impacts That Were "Extremely Important" to Reduce by TD Participants**



Regardless of transfusion status, approximately 60% of participants reported frequent transfusions as being "extremely" or "quite a bit" bothersome

TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive. [a] Responses are shown in order of descending frequency from left to right. [b] Other includes positive impacts on health, pain to the area, time of transfusion, painful procedure, and schedule. [c] Percentages were calculated per impact out of the number of TD participants (N=76 total) who reported that impact.

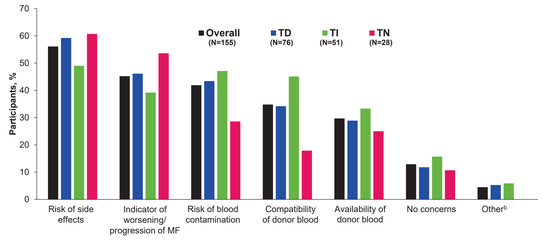
Figure 5: **Reported (A) Inconvenient Aspects of Frequent Blood Transfusions[a,b] and (B) Time Spent at or Traveling to the Transfusion Clinic Among TD Participants**



Overall, 59% of participants reported frequent transfusions to be "extremely" or "quite a bit" inconvenient; time spent at the clinic was cited by the majority as most inconvenient, with more than 50% of participants reporting spending over 3 hours

TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive. [a] Responses are shown in order of descending frequency from left to right. [b] Most common inconvenient aspects are shown.

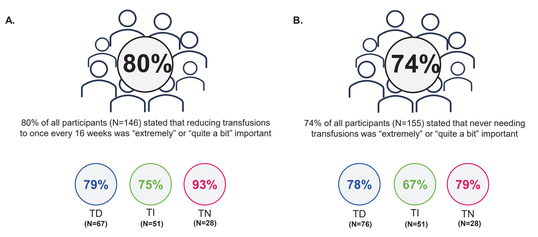
Figure 6: **Concerns or Worries Relating to Blood Transfusions[a]**



The risk of side effects and the need for frequent transfusions being an indicator of worsening or progression of MF were common concerns among all participants

MF, myelofibrosis; TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive. [a] Responses are shown in order of descending frequency from left to right. [b] Other includes "you worry about everything," transportation, pain, and time.

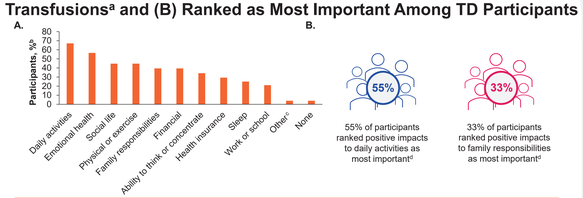
Figure 7: **Proportion Reporting That (A) Reducing the Frequency of Blood Transfusions[a] and (B) Never Needing Blood Transfusions Was "Extremely" or "Quite a Bit" Important**



Participants placed a high level of importance on reducing the frequency of transfusions

TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive. [a] For TD participants, this was a reduction from the individual's current baseline transfusion frequency. Participants were not asked about frequencies that would constitute a worsening from their status. TI and TN participants were asked to assume a baseline of biweekly transfusions; shown all frequency reductions and asked to rate each based on how they anticipated they would feel if they were to have transfusion experience.

Figure 8: **Positive Impacts (A) of Reducing the Frequency of Blood Transfusions[a] and (B) Ranked as Most Important Among TD Participants**



Positive impacts to daily activities and emotional health if they experienced reduced transfusion frequency were anticipated by TD participants

TD, transfusion dependent. [a] Responses are shown in order of descending frequency from left to right. [b] N=76. [c] Other includes fewer doctor visits, better health, and driving time. [d] Percentages are based on the number of participants who had previously reported these positive impacts (daily activities, 28 of 51 reporting participants ranked as most important; family responsibilities, 10 of 33 reporting participants ranked as most important).

Discussion

- Study participants both with and without anemia reported relatively high burden associated with their anemia symptoms, which resulted in experience with or a perception of a range of impacts to daily activities, physical activities or exercise, emotional health, and the ability to think or concentrate
  - Most participants reported treatment to reduce the level of anemia they experience as important
- Regardless of personal experience and dependency on blood transfusions, patients consistently reported significant burden associated with frequent blood transfusions, including impacts to daily activities, emotional health, family responsibilities, social life, finances, and health insurance
  - Most TD participants reported that it would be quite a bit or extremely important to reduce these associated impacts
  - Participants placed increasing levels of importance on reducing the frequency of blood transfusions to varying degrees and high levels of importance on entirely avoiding the need for frequent blood transfusions
- Our findings highlight the potential importance to patients with MF of treatment options that can help achieve and maintain transfusion independence

Conclusion

- Participants with MF view the need for transfusions as a substantial burden to be avoided
- Participants highly value treatments that can mitigate anemia symptoms as well as reduce the frequency of or avoid the need for transfusions

Abbreviations

JAK, Janus kinase; MF, myelofibrosis; MFSAF, Myelofibrosis Symptom Assessment Form; QOL, quality of life; TD, transfusion dependent; TI, transfusion independent; TN, transfusion naive.

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