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Identifying Treatment Patterns for Essential Thrombocythemia Using Real-world Data

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KEY INSIGHTS AND CONCLUSIONS

- A majority of patients (67%) were observed to receive no treatment;
- Treated patients were seen to cycle from hydroxyurea to anagrelide back to hydroxyurea, with hydroxyurea use in 94%, 15% and 51% of patients in 1L, 2L and 3L, respectively, and anagrelide use in 45% and 22% of patients in 2L and 3L, respectively.
- Data suggests therapy switching associated with the cycling of hydroxyurea to anagrelide to hydroxyurea and limited use of other therapies, may indicate a lack of available treatment options for ET patients.

REFERENCES

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ABBREVIATIONS

ET: Essential Thrombocythemia; **HU:** Hydroxyurea;
LOT: Line of therapy

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INTRODUCTION

- Essential thrombocythemia (ET) is a myeloproliferative neoplasm defined by excessive production of thrombocytes (platelets), leading to increased risk of arterial thrombosis, venous thrombosis, and hemorrhagic complications, and potential transformation into aggressive disease such as myelofibrosis or acute myeloid leukemia[1]
- The annual incidence of ET is between 1.2-3.0 per 100,000 population and typically affects patients between the ages of 55 and 65 years[2]
- Current treatment options for ET include aspirin in patients at low risk for thrombosis. Cytoreductive therapies indicated for use in ET populations include hydroxyurea and anagrelide[1,3]
- There is currently limited information on the real-world treatment landscape for ET in the United States, necessitating further research to elucidate treatment patterns

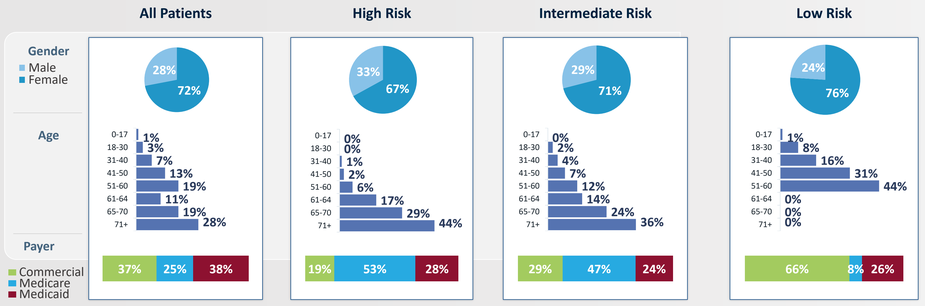
OBJECTIVE

- To characterize the treatment landscape among patients diagnosed with ET in a US healthcare population

METHODS

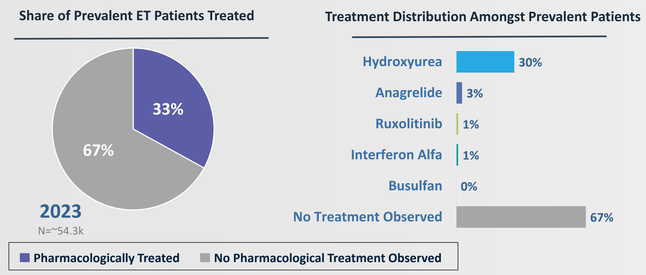
- This analysis utilized Veeva Compass claims data, covering more than 300 million patients in the United States
- Patients with ET diagnoses were identified as any patient with 2 or more diagnosis claims for essential thrombocythemia (ICD-10 D47.3)* at least 30 days apart anytime between 2020 - 2023 ("study timeframe"), with at least 1 diagnosis occurring within one year from index date (index date was defined as the first ET diagnosis observed in the study period)
- Patients were also required to have 1 or more medical claims and 1 or more prescription claims present within one year from index date, in order to proxy for continuous enrollment
- Patients were excluded if they had any of the following:
  - 2 or more diagnosis claims for secondary thrombocytosis or hereditary thrombocytosis anytime before or after the initial ET diagnosis
  - Two or more diagnosis claims for polycythemia vera, chronic myelogenous leukemia, or myelodysplastic syndrome 6 months before or after the initial ET diagnosis
  - 2+ Dx claims for AML or MF anytime before the initial ET diagnosis
- For the line of therapy analysis, the following rules were implemented:
  - 7 days minimum exposure required to qualify as formal treatment line
  - New treatment lines were defined as switch in drug class (discontinuation and restart of the same drug will be considered the same line)
  - Addition of new product within 30 days of initiating former product would be considered combination treatment of the same line
  - Addition of "product B" more than 30 days after initiation of "product A" considered a line advancement

Figure 1 | Demographics of ET Patient Cohort



- **Gender skews towards more females**, aligned with prior available literature[4]
- **Over 80% of patients are over the age of 40**, reflecting the median age of onset of 58 years in the ET population[1]
- A majority (47%-53%) of intermediate and high-risk patients were associated with Medicare Insurance

Figure 2 | Treatment Amongst Prevalent Patients



- **Treatment rate amongst ET prevalent patients was 33% in 2023**, indicating low rates of pharmaceutical intervention even among prevalent patients
- **Hydroxyurea was the most common treatment observed among all patients** with 30% of prevalent patients
- **Use of more advanced treatments (i.e., anagrelide, ruxolitinib, interferon alfa, and busulfan) was low**, with anagrelide being the most frequent

Figure 3 | Cytoreductive Treatment Choice Among Treated Patients

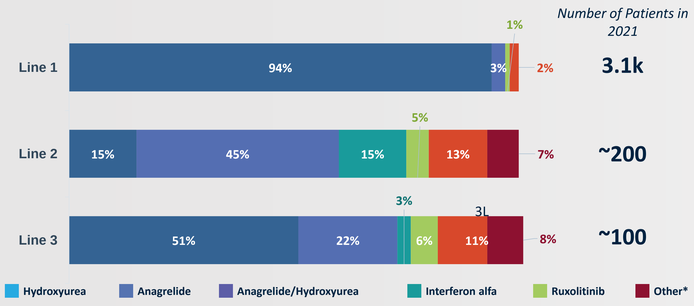
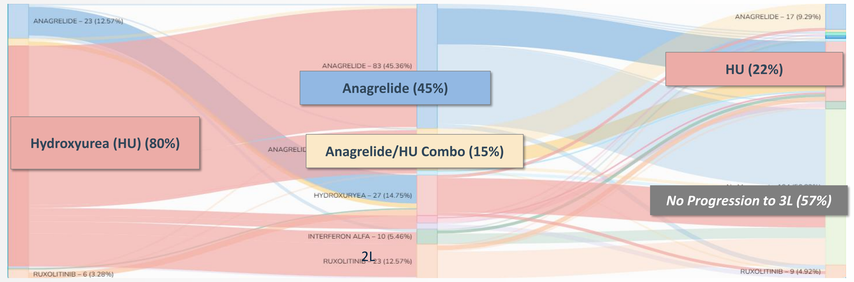
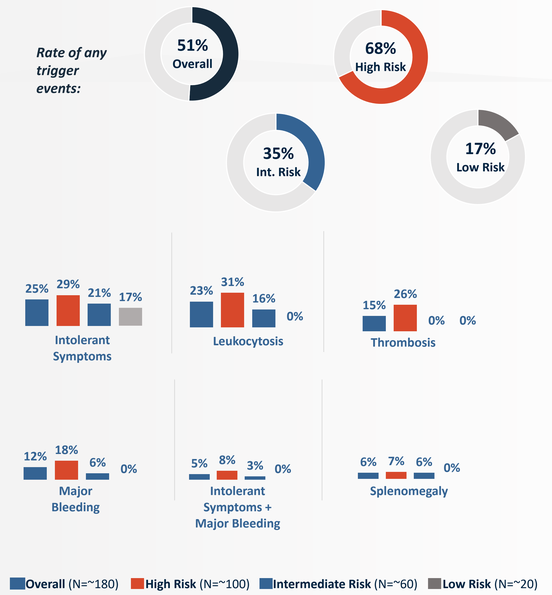


Figure 4 | Treatment Dynamics Among Patients with ≥1 Treatment Switch



- **Among patients treated with hydroxyurea in 1L, ~5% switched and progressed to 2L**
- **The most common switches were to anagrelide (45% of 2L therapies)**, followed by anagrelide/hydroxyurea combination therapy (15%)
- **A subset of patients switch back to hydroxyurea (22% of 3L therapies)**
- **Mean number of treatment switches was 1.1 (median: 1)** (data not in figure)

Figure 5 | Trigger Events Within 3 Months to Treatment Switch



- **Over half (51%) of prevalent patients experienced a trigger event** within 3 months of treatment switch
- **Intolerant symptoms** (presence of fever, nausea, vomiting, mouth sores or leg ulcers), **leukocytosis and thrombosis were the most prevalent trigger events**

DISCUSSION

- Most diagnosed patients with ET remain untreated with cytoreductive therapy
- For patients who are treated with cytoreductive therapy, a subset switch treatment following a trigger event such as leukocytosis or thrombosis, indicating unmet needs with current treatments to prevent these events
- Among patients who are treated with cytoreductive therapy, hydroxyurea is the most common; however, a majority of patients experience at least 1 switch during their treatment journey, and patient cycling is seen for patients from hydroxyurea to anagrelide back to hydroxyurea, indicating a lack of adequate treatment options

LIMITATIONS

- Due to the nature of claims data, this analysis can only account for patients who were continuously enrolled and receiving care represented by claims; therefore the patient sample may not be representative of all patients with ET
- Line of therapy rules may not accurately represent all true advancements in treatment

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