Real-world Experience with Fostamatinib for the Treatment of Immune Thrombocytopenia (ITP): Patient-reported Outcomes

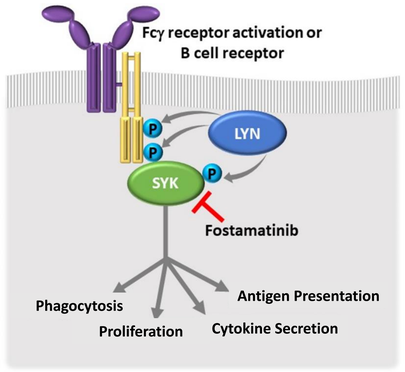
Deon Wolliston, APRN, MSN, PhD[1]; John Hales, PhD[1]; Lucas Buxman, MBA[2]; Leslie K Todd[1]

[1]Rigel Pharmaceuticals Inc, South San Francisco, CA, USA; [2]Claritas Rx, Inc, San Francisco, CA, USA.

Background

- Fostamatinib is an orally administered spleen tyrosine kinase (SYK) inhibitor indicated for the treatment of thrombocytopenia in adults with chronic ITP who had an insufficient response to previous therapy[1].
- SYK inhibition prevents platelet phagocytosis by Fcγ receptors on macrophages and may also prevent antibody production by B cells (**Figure 1**).
- In the phase 3 clinical trial program, fostamatinib demonstrated durable efficacy for patients with ITP[2-5].

Figure 1. Fostamatinib and the SYK-mediated pathway



Introduction and Objectives

- Data from randomized-controlled trials do not necessarily represent expected outcomes in real-world experience[6,7] due to:
  - Constraints of protocols
  - Strict enrollment criteria that may limit external validity
- Real-world data (RWD) incorporate clinical experience, patient preferences, and real-world issues and may complement results obtained from randomized-controlled trials.
  - The US FDA is evaluating the use of real-world evidence for regulatory decision-making[8].
- Several case series have been published demonstrating real-world experience with fostamatinib[9,10].
- Previous studies have used patient-reported outcomes, including patient-reported clinical data, to evaluate ITP treatment[11].
- The objective of this analysis was to provide real-world patient-reported outcomes from patients with ITP treated with fostamatinib who obtained support through RIGEL ONECARE®.

Methods

Inclusion Criteria

- ICD10 code consistent with ITP diagnosis
- ≥1 pre-treatment platelet count reported by the healthcare provider +/- patient
- ≥1 post-fostamatinib initiation platelet count reported by the patient
- ≥3 months of drug supply with ≤60-day gap in drug supply during the treatment period
- Provided consent to share data

Endpoints

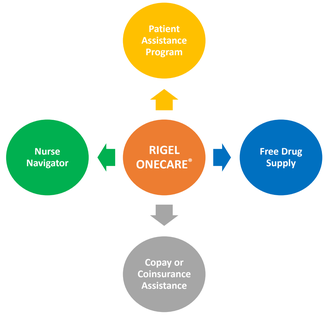
- Median post-treatment platelet counts
- Proportion of patients who achieved ≥30,000/μL and ≥50,000/μL at least once
- Proportion of patients with ≥30,000/μL and ≥50,000/μL on ≥50% of post-treatment readings
- Proportion of patients with ≥1 post-treatment platelet count doubled from pre-treatment median

Overview of Patient Support Program

Rigel OneCare® is sponsored by Rigel Pharmaceuticals and offers service and support to patients prescribed fostamatinib (**Figure 2**). This program includes:

- A nurse navigator who may provide resources and support for the insurance coverage process, promote medication adherence, answer questions, and connect patients to their provider when appropriate
- Nurse navigators call patients every two weeks, with schedule adjustments as requested by the patient, and may collect patient-reported platelet counts
- Affordability and access programs such as copay/coinsurance assistance and free drug supply in cases where patients meet program criteria

Figure 2. Rigel OneCare® Services



* All Rigel programs are subject to eligibility requirements. Support programs are only available for patients that are at least 18 years old, residents of the USA or Puerto Rico, and have an on-label indication. Full program criteria are not displayed and can be provided by Rigel OneCare. Restrictions apply. Rigel OneCare is not intended to provide medical advice, replace prescribed treatment plans, or provide treatment or case management services. RIGEL ONECARE is a patient support center sponsored by Rigel Pharmaceuticals, Inc. Patients are advised to always talk to their healthcare provider about any medical decisions.

Results

- As of the data cutoff date (10 March 2022), 318 patients met the eligibility criteria and were included in the analysis (**Table 1**).
- 108 (34%) patients remained on the 100 mg BID regimen throughout treatment, and 210 (66%) increased their dose to 150 mg BID during treatment.
- Median pre-treatment platelet count was 32,000/μL and median post-treatment count was 68,800/μL (**Figure 3**).
- After initiating therapy with fostamatinib:
  - 269 (85%) achieved one or more platelet counts of ≥30,000/μL (**Figure 4**).
  - 238 (75%) had at least half of their post-treatment counts ≥30,000/μL (**Figure 4**).
  - 244 (77%) had one or more platelet counts of ≥50,000/μL (**Figure 4**).
  - 201 (63%) had at least half of their post-treatment counts ≥50,000/μL (**Figure 4**).
  - 207 (65%) patients had ≥1 post-treatment platelet count doubled from their pre-treatment median.

Table 1. Baseline Characteristics and Overview of Fostamatinib Treatment

Characteristic	Fostamatinib-treated patients N=318
Age, years Median (range)	66.9 (19-100)
Sex, n (%) Female Male	184 (57.9) 134 (42.1)
Mean duration of fostamatinib therapy, months	10.9
Pre-treatment platelet count, % of patients ≤15,000/μL >15,000-30,000/μL >30,000-50,000/μL >50,000/μL	 25.2 23.6 18.2 33.0
Mean number of platelet counts available: Pre-treatment Post-treatment	 2.1 6.7

Results

Figure 3. Change in Median Platelet Counts Throughout Treatment with Fostamatinib (N=318)

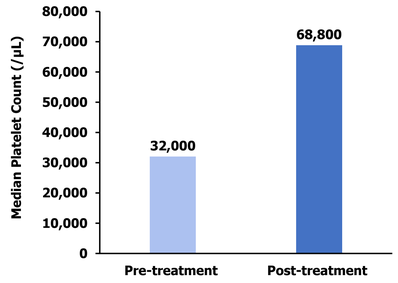
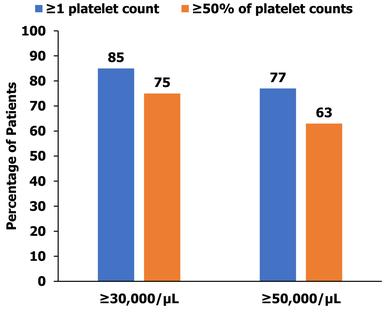


Figure 4. Proportion of Patients with Platelet Response (N=318)



Discussion

- In this analysis, median pre-treatment platelet counts were higher than those observed in randomized-controlled trials (RCT), possibly due to patients receiving rescue treatment or switching from other maintenance therapies without the washout period required in a clinical trial.
- As in the RCTs, most patients achieved platelet counts ≥30,000/μL at least once, but this RWD demonstrated higher proportions of patients with counts ≥50,000/μL,[2-5] highlighting differences between RCTs and real-world experience.
- Most patients (65%) demonstrated a doubling of their median pre-treatment platelet count.
- This report did not evaluate safety. Additionally, the potential for reporter bias exists, since patients self-reported post-treatment platelet counts. Pre-treatment platelet counts were reported by the healthcare provider during enrollment and may have been supplemented by patient-reported data.

Conclusions

- In this analysis of real-world data, fostamatinib demonstrated effectiveness. These results complement those of the clinical trial program and further support the use of fostamatinib for management of ITP.

References

1. Rigel Pharmaceuticals, Inc. TAVALISSE® (fostamatinib disodium hexahydrate) [prescribing information]. San Francisco, CA: Rigel Pharmaceuticals, Inc.; 2020 November.
2. Bussel J, et al. *Am J Hematol.* 2018;93(7):921-30.
3. Bussel J, et al. *Am J Hematol.* 2019;94(5):546-53.
4. Boccia R, et al. *Br J Haematol.* 2020;190(6):933-8.
5. Cooper N, et al. *Ther Adv Hematol.* 2021;12:1-12.
6. Saturni S, et al. *Pulm Pharmacol Ther.* 2014;27(2):129-38.
7. Williamson J, Lloyd M. *J Prescrib Pract.* 2019;1(5):247.
8. Concato J, et al. *Pharmacoepidemiol Drug Saf.* 2020;29:1514-17.
9. Mehta AR, et al. *Acta Haematol.* 2021 Dec;14:1-8.
10. Hughes DM, et al. *J Adv Pract Oncol.* 2021 Jul;12(5):508-517.
11. Brown TM, et al. *BMC Blood Disord.* 2012 Mar 22;12:2.

Acknowledgments

Professional medical writing was provided by Sandra M. Aguero, PharmD, BCPS, of PharmaWrite, LLC (Princeton, NJ, USA), and was funded by Rigel Pharmaceuticals, Inc

Disclosures

DW, JH, LKT: Employee, Stock ownership, Rigel Pharmaceuticals, Inc
LB: Employee, Claritas, LLC

Presented at the 27th Annual International Congress of Hematologic Malignancy • Miami, FL • February 23-26, 2023