INTRODUCTION

Immune Thrombocytopenia (ITP)

- ITP is characterized by immune-mediated platelet destruction and impaired function of platelet production, leading to thrombocytopenia, a pathological state, and a direct impact on quality of life.
- Current therapies for adults include IVIG, corticosteroids (CS), or TPO receptor agonists (TPO-RA).
- CS is relatively safe and effective but requires lifelong treatment.
- TPO-RA may cause a rapid increase in platelet counts/thrombosis risk.
- Platelet responses are generally transient, and patients usually require further treatment.
- Platelet responses were consistent overall and across endpoints. Stable responders on concomitant therapy (n=13/31).

Rilzabrutinib does not alter platelet aggregation in blood taken from healthy volunteers or patients with ITP. Platelet aggregation was assessed using light transmission from healthy volunteers or patients with ITP. Platelet counts at study entry were < 30,000/μL.

AIMS

- To present the results from a phase I/II study of rilzabrutinib in patients with relapsed/refractory, primary or secondary ITP who had no other available treatment options.

METHODS AND PATIENTS

- Patients were heavily pretreated, including patients escalated to 400 mg bid (platelet counts ≥ 30 × 10⁹/L).
- Rapid onset was seen in 53% of patients who initiated rilzabrutinib at 400 mg bid (platelet counts ≥ 30 × 10⁹/L) by the first week of treatment.
- Stable responses were achieved in 28% of patients on concomitant therapy (n=13/31).
- Patients were heavily pretreated, including patients escalated to 400 mg bid (platelet counts ≥ 30 × 10⁹/L).
- Baseline platelet counts were similar across the arms.

RESULTS

- Platelet responses were maintained in the majority of patients who switched to rilzabrutinib (9/13).
- Platelet counts were ≥ 50 × 10⁹/L in 42% (33, 55) of responders, ≥ 30 × 10⁹/L in 44% (31, 55) of responders, and ≥ 20 × 10⁹/L in 50% (38, 62) of responders.
- Platelet responses were consistent overall and across endpoints. Stable responders on concomitant therapy (n=13/31).
- Baseline platelet counts were similar across the arms.

CONCLUSIONS

- Fifty percent of patients achieved the primary endpoint response when initiated on rilzabrutinib 400 mg bid and treated for 12 weeks.
- Rapid onset was seen in 53% of patients who initiated rilzabrutinib at 400 mg bid platelet counts ≥ 30 × 10⁹/L.
- Durable responses were achieved in the majority of responding patients (21% of responders). Platelet counts were ≥ 50 × 10⁹/L in 42% (33, 55) of responders, ≥ 30 × 10⁹/L in 44% (31, 55) of responders, and ≥ 20 × 10⁹/L in 50% (38, 62) of responders.

REFERENCES


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CORRESPONDING AUTHOR

David Kuter
Email: Dkuter@mgh.harvard.edu

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