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Eltrombopag, Low-Dose Rituximab, and High-Dose Dexamethasone Combination for Patients with Newly Diagnosed Immune Thrombocytopenia: A Pilot "Total Therapy" Study

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Abstract

Introduction: Immune thrombocytopenia (ITP) is an autoimmune disorder that results from platelet destruction and production suppression. Frontline-therapy includes corticosteroids, intravenous immune globulin or anti-D immunoglobulin. Single-agent treatments have not been successful in inducing prolonged remission, as relapse will occur in approximately 50% of patients. Low-dose rituximab (100 mg) has been used for the treatment of ITP, showing an activity almost similar to the 375 mg/m^2 standard dose. We and others have reported sustained response rates ranging from 58% to 76% using rituximab plus dexamethasone as a frontline therapy. Eltrombopag is a thrombopoietin nonpeptide mimetic that has been shown to raise platelet count in chronic ITP, and we have previously reported eltrombopag/ dexamethasone as a feasible frontline therapy for ITP reaching 100% response rates.The lack of sustained response in many adult patients with newly diagnosed acute ITP has stimulated the search for a treatment that modifies the natural course of the disease.

Objetive: We aim to evaluate efficacy, safety, and response duration of low-dose weekly rituximab (100 mg weekly, four doses) plus high-dose dexamethasone (40 mg PO, days 1-4) in combination with eltrombopag (50 mg, days 1-28) as frontline therapy in newly diagnosed primary ITP in an ambulatory setting.

Methods: This is an ongoing open-label, single-arm study performed in patients with newly diagnosed ITP from the Hospital Universitario Dr. Jose Eleuterio Gonzalez in Monterrey, Mexico (Clinical trials.gov NCT02834286). Eligible patients are 16 years or older, with bleeding manifestations and/or a platelet count ²30×10^9/L, without previous treatment. Patients are excluded if they had active infection, pregnancy, or a malignant disease. A complete blood count is performed at baseline, on days 3, 5, 7 and then weekly for 28 days, monthly until month 6, and every 3 months thereafter. Partial and complete responses are defined as an increase in platelet counts ³30×10^9/L and ³100×10^9/L, respectively.

Results: Ten consecutive patients have been enrolled from March 2015 until July 2016. Median age was 37 years (16-61). Six patients were women (60%) and four were men (40%). Median platelet account at diagnosis was 7 « 10^9/L (range 1.2-28). Median follow-up has been 7 months (range 1-13). All patients achieved at least a partial response (PR) at a median of 4 days (range 3-14). Complete response (CR) was achieved in 9 patients in a median of 7 days (7-22); all of them were still in CR at the end of treatment (Day 28). One patient lost response at 28 days and received a second high-dexamethasone course maintaining CR. No significant adverse effects have occurred during treatment, only 1 patient reported mild myalgia. No relapses have been documented until now.Currently, 8 patients remain in CR and 2 in PR.

Conclusion: This is the first trial evaluating the response of low-dose rituximab in combination with eltrombopag and high-dose dexamethasone in newly diagnosed patients with ITP.Low-dose rituximab in combination with eltrombopag and high dose dexamethasone is a feasible frontline therapy for ITP. This drug combination showed high response rates achieved very rapidly, with a low incidence of side effectsand might represent an attractive option in patients with ITP and substantial bleeding.

Table**Characteristics and follow-up of patients**M: Male, F: Female, CR: Complete Response, PR: Partial Response

Patient	Gender/ Age	Basal platelets 10^9/L	Best Response	Time to partial response (days)	Time to complete response (days)	Follow-up (months)	Current status
1	M/21	11	CR	6	11	13	CR
2	M/50	6	CR	7	14	9	CR
3	F/61	2	CR	3	7	8	CR
4	F/54	5	CR	5	7	8	CR
5	F/16	28	CR	3	7	8	CR
6	F/26	4	CR	5	22	6	CR
7	M/57	12	CR	3	7	6	PR
8	F/40	1.22	PR	4	-	4	PR
9	F/26	4	CR	4	7	1	CR
10	M/34	12	CR	3	7	4	CR

Disclosures

Gomez-Almaguer: *Amgen:* Consultancy, Membership on an entity's Board of Directors or advisory committees; *Celgene:* Consultancy, Membership on an entity's Board of Directors or advisory committees; *Janssen:* Consultancy, Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; *Bristol:* Consultancy, Membership on an entity's Board of Directors or advisory committees.

Author notes

*Asterisk with author names denotes non-ASH members.