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Original Research Article

Safety and Effectiveness of Tofacitinib in Biologic-Naïve Indian Patients with Moderate-to-Severe Ulcerative Colitis: Real-World Experience from Northern India

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Conflict of interest: Nil

Abstract:

Background: Tofacitinib, an oral Janus kinase (JAK) inhibitor, offers an advanced small-molecule option for moderate-to-severe ulcerative colitis (UC). Despite proven efficacy in pivotal trials, Indian real-world data remain scarce. This study aimed to evaluate the safety and effectiveness of tofacitinib in biologic-naïve Indian patients treated under routine care at a tertiary center in Jaipur.

Methods: A retrospective observational study was performed on 50 adult, biologic-naïve UC patients receiving tofacitinib (10 mg BID × 8 weeks, followed by 5–10 mg BID maintenance) between January 2022 and June 2024 at Apex Hospital, Jaipur. Patients were followed for 52 weeks. Primary outcomes included clinical remission, clinical response, endoscopic mucosal healing, and steroid-free remission. Safety events were collected from records.

Results: Mean age was 38 ± 10 years; 60% were male. Baseline disease was moderate in 70% and severe in 30%. Clinical remission rates at 8, 16, 24, and 52 weeks were 40%, 48%, 56%, and 54%, respectively; clinical response rates were 75%, 85%, 70%, and 78%. Endoscopic mucosal healing occurred in 55% and 45% at 16 and 52 weeks, respectively. Steroid-free remission was achieved in 50% at 52 weeks. Adverse events occurred in 35%, mostly mild; two serious events (cytomegalovirus colitis and anal abscess) resolved completely.

Conclusion: Tofacitinib demonstrated favorable effectiveness and safety in biologic-naïve Indian UC patients. Real-world outcomes mirrored international data, supporting its role as a cost-effective oral advanced therapy in India.

Keywords: Tofacitinib; Ulcerative Colitis; Janus Kinase Inhibitor; Inflammatory Bowel Disease, Chronic Inflammatory Disease.

Introduction

Ulcerative colitis (UC) is a chronic inflammatory disease of the colon characterized by relapsing mucosal inflammation, rectal bleeding, diarrhea, and urgency [1,2]. Historically considered rare in developing countries, UC incidence and prevalence have risen markedly across South Asia over the last two decades [3–5]. Recent meta-analyses show a steady increase in inflammatory bowel disease (IBD) across India, paralleling urbanization, western dietary patterns, and environmental change [6–8].

The Indian IBD registry, along with multicentric studies, highlights a growing disease burden, particularly in the northern and western regions [9–11]. The registry revealed significant geographic variation and an increasing proportion of patients presenting with moderate-to-severe disease at diagnosis, longer steroid exposure, and early steroid dependence [12,13].

Standard therapy for moderate-to-severe UC includes aminosalicylates, corticosteroids, and immunomodulators (azathioprine or 6-mercaptopurine) [14]. Biological agents such as anti-TNF inhibitors (infliximab, adalimumab), vedolizumab, and ustekinumab have significantly improved remission and mucosal healing rates globally [15,16]. However, in India, barriers including cost, infusion logistics, and lack of insurance coverage limit biologic utilization [17]. Oral small-molecule inhibitors like tofacitinib offer a feasible, cost-effective alternative [18].

Tofacitinib inhibits JAK1/JAK3 signaling, suppressing multiple cytokines implicated in UC pathogenesis. Its oral administration, rapid onset, and reversibility make it a practical option for Indian patients. Large global trials (OCTAVE Induction, Sustain) demonstrated its efficacy for induction and maintenance of remission [18]. However, due to India's higher latent tuberculosis and viral infection

rates, local data are essential for safety validation. Moreover, most published Indian experiences combine biologic-experienced and naïve cohorts; hence, this study focuses exclusively on biologic-naïve Indian UC patients treated with tofacitinib in a real-world setting.

Methods

Study design and setting: A retrospective, single-center observational study was conducted at Apex Hospital, Jaipur, India, between January 2022 and June 2024. The study population, treatment protocol, follow-up schedule and outcome assessment were derived from hospital records.

Participants (Inclusion and exclusion criteria)

Inclusion criteria

- Adults (≥18 years) with a diagnosis of ulcerative colitis confirmed by clinical, endoscopic and histologic criteria.
- Moderate-to-severe disease activity (Mayo score ≥6) at baseline.
- Biologic-naïve patients who received tofacitinib during the study period.
- Minimum recorded follow-up of 52 weeks or until treatment discontinuation.

Exclusion criteria

- Prior exposure to biologic agents for inflammatory bowel disease.
- Concomitant participation in other interventional clinical trials during the observation period.
- Incomplete records preventing assessment of primary outcomes.

Baseline evaluation and pre-treatment screening: Baseline data collected included demographics (age, sex), disease duration and extent, baseline Mayo score, prior therapies (5-ASA, corticosteroids, thiopurines), comorbidities, body mass index, smoking status, and latent infection screening. All patients underwent pre-treatment screening for latent tuberculosis and standard viral markers as per institutional protocol; latent TB was treated prior to initiating tofacitinib when indicated.

Treatment protocol: Tofacitinib was administered as per routine clinical practice: induction with 10 mg twice daily for 8 weeks, followed by maintenance dosing of 5–10 mg twice daily based on clinical response and treating physician discretion. Concomitant medications (aminosalicylates, corticosteroids, immunomodulators) were used and tapered according to standard clinical practice.

Outcome measures and definitions

Primary effectiveness outcomes:

- Clinical response and clinical remission at weeks 8, 16, 24 and 52.
- Endoscopic mucosal healing at 16 and 52 weeks.
- Steroid-free remission at 52 weeks.

Definitions used in the study followed standard clinical practice and were applied consistently during record review (e.g., clinical remission = symptom resolution consistent with Mayo scoring criteria as recorded in charts).

Safety assessment: Adverse events (AEs) and serious adverse events (SAEs) occurring during follow-up were recorded from case files and electronic records. Specific events of interest included infections (herpes zoster, TB reactivation, cytomegalovirus), thromboembolic events, major adverse cardiovascular events (MACE), malignancy, laboratory abnormalities (anemia, dyslipidemia), and UC flares. All AEs were categorized by severity and outcome as documented in the records.

Data collection and management: Data were abstracted into a predesigned spreadsheet from hospital case sheets and electronic medical records. Key time points documented were baseline and weeks 8, 16, 24 and 52. Missing or ambiguous entries were cross-checked with treating clinicians or source records when possible. Patient identifiers were removed and data were analyzed in de-identified form.

Statistical analysis: Data were analyzed using descriptive statistics. Continuous variables were expressed as mean ± standard deviation, and categorical variables as frequency and percentage. Statistical analysis was performed using Microsoft Excel 2019.

Ethical approval: The study was conducted in accordance with the Declaration of Helsinki and institutional guidelines. Institutional Ethics Committee approval was obtained before data collection.

Results

Mean age was 38 ± 10 years; 60% were male. Baseline disease severity was moderate in 70% and severe in 30%. Clinical remission was achieved in 40%, 48%, 56%, and 54% at 8, 16, 24, and 52 weeks, respectively. Clinical response rates were 75%, 85%, 70%, and 78%. Endoscopic mucosal healing was seen in 55% at 16 weeks and 45% at 52 weeks. Steroid-free remission was achieved in 50% at 52 weeks. AEs occurred in 35%, mainly mild anemia, dyslipidemia, and UC flare. Two SAEs (cytomegalovirus colitis, anal abscess) resolved completely. No herpes zoster, tuberculosis

reactivation, malignancy, MACE, or VTE was reported.

Table 1: Baseline demographic and clinical characteristics (n = 50)

Characteristic	Value
Mean ± SD age (years)	38 ± 10
Male : Female ratio	1.5 : 1 (60% : 40%)
Median disease duration (years)	4 (2–8)
Disease extent	Proctosigmoiditis 16%, Left-sided 48%, Pancolitis 36%
Baseline Mayo score (mean ± SD)	8.1 ± 1.6
Severity	Moderate 70%, Severe 30%
Previous 5-ASA exposure	100%
Previous corticosteroid exposure	90%
Previous thiopurine use	40%
Latent TB treated before start	20%
Comorbidities	HTN 12%, Diabetes 8%
Smoking (current)	6%
Mean BMI (kg/m²)	23.4 ± 3.1

Table 2: Clinical and endoscopic outcomes of tofacitinib therapy

Time point (weeks)	Clinical response n (%)	Clinical remission n (%)	Endoscopic healing n (%)	Steroid-free remission n (%)
8	38 (75%)	20 (40%)	—	10 (20%)
16	43 (85%)	24 (48%)	22 (55%)	18 (36%)
24	35 (70%)	28 (56%)	19 (48%)	23 (46%)
52	39 (78%)	27 (54%)	15 (45%)	25 (50%)

Table 3: Adverse events observed during follow-up (n = 50)

Adverse Event	n (%)	Severity	Outcome
Anemia	5 (10%)	Mild	Resolved
Dyslipidemia	4 (8%)	Mild	Controlled
UC flare	4 (8%)	Moderate	Steroid responsive
Cytomegalovirus colitis	1 (2%)	Severe	Recovered
Anal abscess	1 (2%)	Severe	Recovered
Headache/Myalgia	3 (6%)	Mild	Resolved
Total patients with ≥1 AE	18 (36%)	—	—
Serious AEs	2 (4%)	—	—

Figure 1. Trend of clinical remission and steroid-free remission over 52 weeks

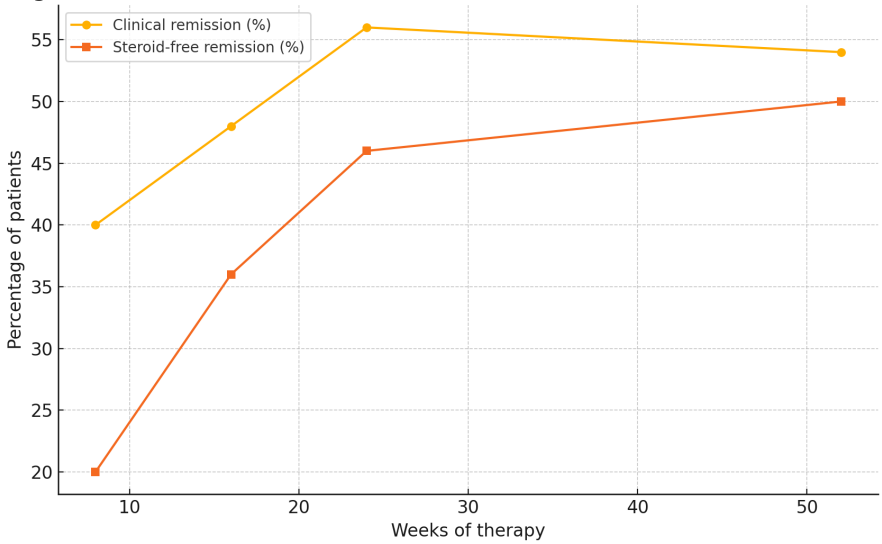

Figure 1: Trend of clinical remission and steroid-free remission over 52 weeks

Line chart showing improvement in clinical and steroid-free remission rates over 52 weeks of tofacitinib therapy.

Discussion

This real-world Indian study demonstrates that tofacitinib is effective and safe in biologic-naïve UC patients, yielding remission and mucosal healing rates comparable to international data [19,20]. Our findings align with Korean post-marketing data and the OCTAVE program, which reported similar long-term outcomes [21,22]. The slightly higher remission rates may reflect the inclusion of biologic-naïve patients, who typically respond better than biologic-experienced individuals [23].

In the Indian context, these findings are significant. UC prevalence and healthcare costs are increasing, yet access to biologic therapy remains limited due to economic and infrastructural constraints [24]. Generic tofacitinib, now available in India, reduces monthly treatment costs to nearly one-fifth of biologics, allowing broader patient access [25]. Furthermore, the drug's oral route eliminates infusion-associated logistics, a major barrier in low-resource settings [26].

Safety findings were favorable, with no tuberculosis reactivation or herpes zoster infection [27]. This is particularly reassuring given India's endemic TB prevalence. Pre-treatment latent TB screening and appropriate prophylaxis were key preventive measures [28]. Mild anemia and dyslipidemia were manageable with standard monitoring. These outcomes reinforce that with structured pre-screening and follow-up, tofacitinib can be safely implemented in Indian clinical practice [29].

Our results echo global evidence supporting JAK inhibitors as fast-acting, reversible, and immunogenicity-free alternatives to biologics [30]. Moreover, tofacitinib's short half-life allows rapid cessation in case of infection—a crucial feature in high-infection-burden countries like India. Comparative data suggest lower infection rates in younger, biologic-naïve cohorts typical of Indian UC demographics.

Cost-effectiveness and practical accessibility are key differentiators in India. Studies have shown that high biologic costs contribute to under-treatment and non-adherence in up to 40% of Indian UC patients [25]. Tofacitinib's affordability may thus address this therapeutic gap. However, long-term Indian pharmacovigilance studies are warranted to assess cardiovascular and thromboembolic risks, especially in older or comorbid populations [29,30].

Limitations include retrospective design, single-center setting, and absence of biochemical endpoints such as CRP or fecal calprotectin. Despite these, our study reflects real-world outcomes and supports tofacitinib's role as an effective and affordable oral therapy for Indian UC patients.

Conclusion

Tofacitinib demonstrated substantial clinical and endoscopic effectiveness with acceptable safety in biologic-naïve Indian patients with moderate-to-severe UC. These findings, consistent with global evidence, support tofacitinib as a practical, oral, and cost-effective treatment option in India. Larger multicentric studies and registry-based surveillance are needed to define long-term outcomes.

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