

Are patient-reported outcomes of advanced therapies adequately measured in the real-world settings in ulcerative colitis?



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BACKGROUND

Context: Ulcerative colitis (UC) is a chronic inflammatory condition that affects the colon and rectum to variable extent. The global burden of disease of UC in 2023, was 5 million.[1] The main symptoms of UC are bloody diarrhoea, urgency, tenesmus, and lower abdominal pain.[2,3] Given that clinical symptoms in UC causes significant patient burden, they are commonly evaluated in clinical trials. Several advanced therapies have become available for patients with moderate-to-severe-UC. However, there is limited evidence on their patient-reported outcome measures (PROMs) in real-world setting.

Aim: To analyze the use of PROMs for advanced therapies in moderate to severe UC in real-world settings.

METHODS

A systematic literature review was conducted in Embase and MEDLINE to identify the real-world studies reporting PROMs on advanced therapies in moderate-to-severe UC patients.

Advanced therapies comprised biologics (i.e., infliximab, adalimumab, golimumab, vedolizumab, ustekinumab) and small molecules (i.e., tofacitinib, filgotinib, upadacitinib, ozanimod).

The key words for searches included terms related to disease, therapies, and study designs, which were combined using Boolean operators.

Studies published in English as full papers (Jan-2012–April-2023) or conference abstracts (Jan-2019–April-2023) were included.

Data extraction parameters:

- Patient reported outcomes (SIBDQ, EQ-5D, PROMIS, PhGA, SF-36)
- Clinical trial characteristics
- Treatment and trial arm

The review followed PRISMA guidelines. One reviewer extracted data, which was checked by second reviewer to ensure quality and accuracy. All data were analyzed quantitatively and qualitatively.

Only a few real-world studies of advanced therapies reported the use of PROMs in moderate-to-severe UC. However, the publications on PROMs in UC have seen a noticeable increase since 2021.

The restricted use could be ascertained to the limited or no guidelines on PROMs assessment in real-world setting.

In routine clinical practice, combining these PROMs with physician’s assessment to integrate disease activity profile of patients might be helpful for precise decision-making in UC care.

Figure 1. Included studies by publication year

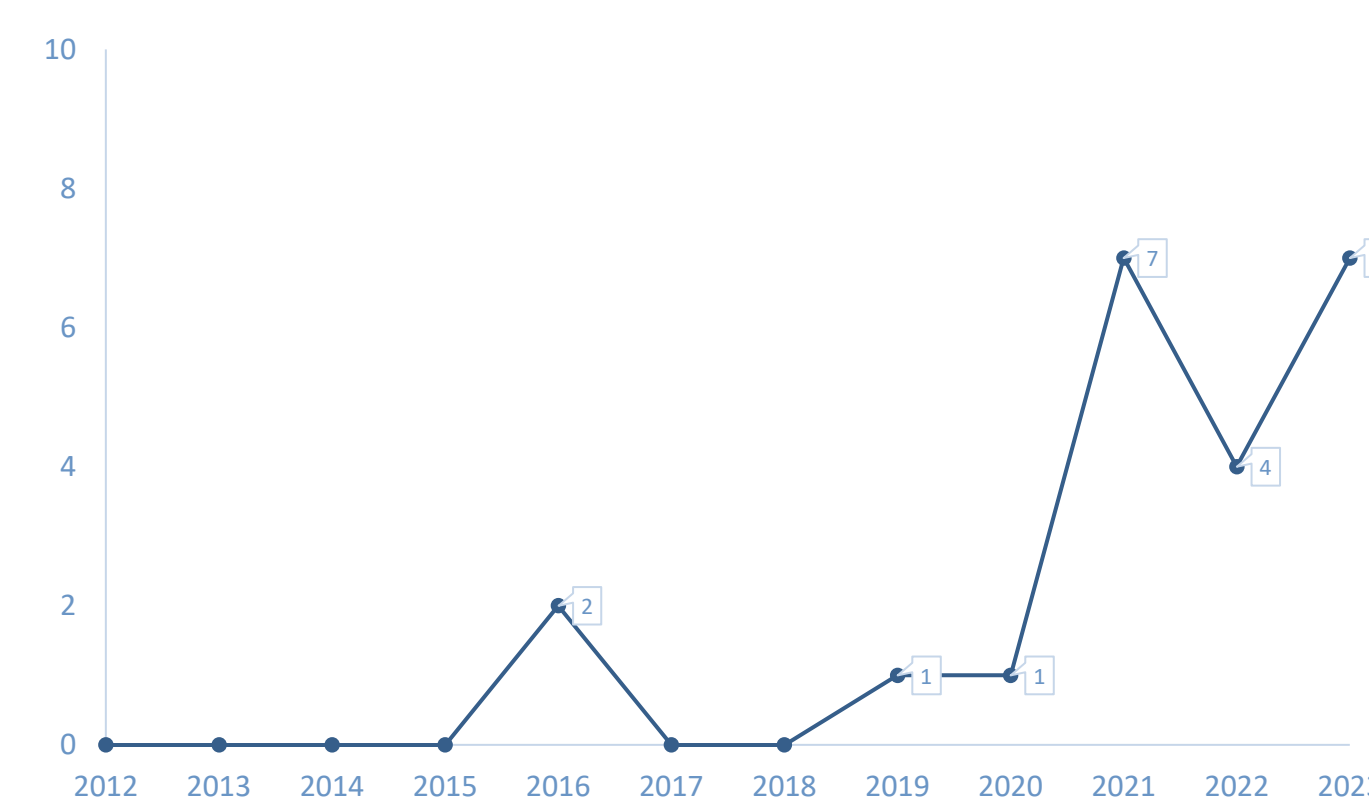


Figure 2. Distribution of studies by geography and country

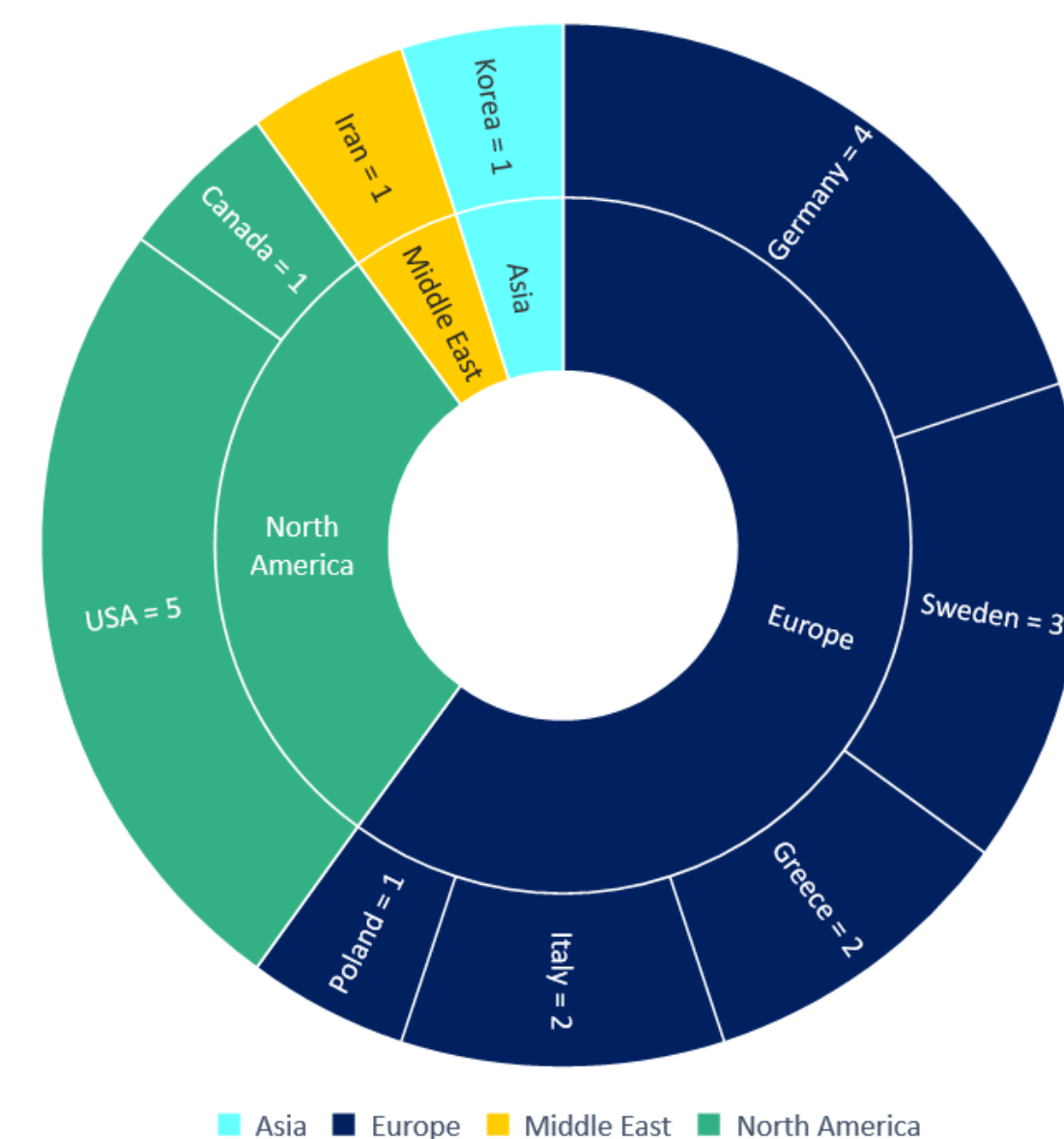


Figure 3. Population among included studies

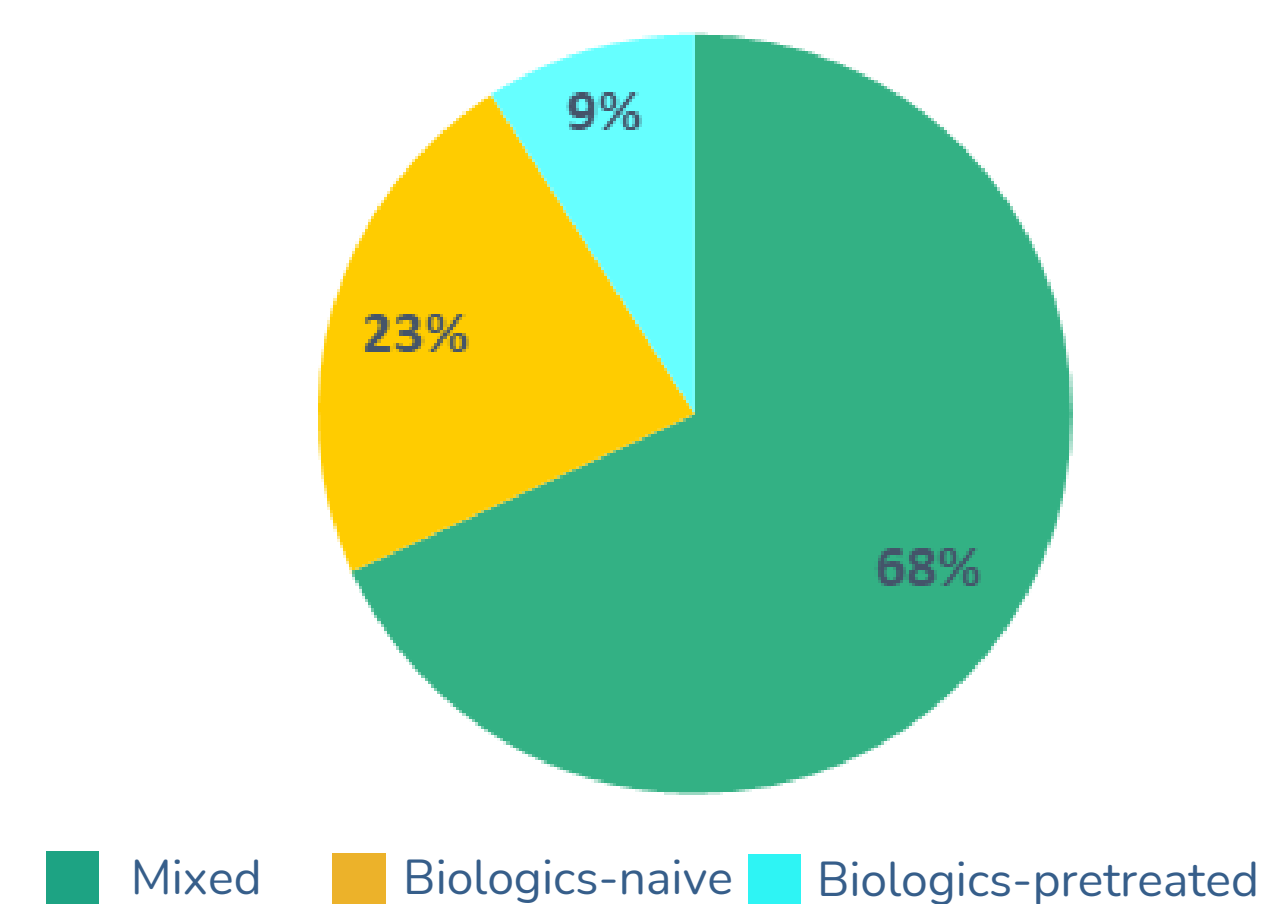


Figure 4. Treatments evaluated in single-arm studies

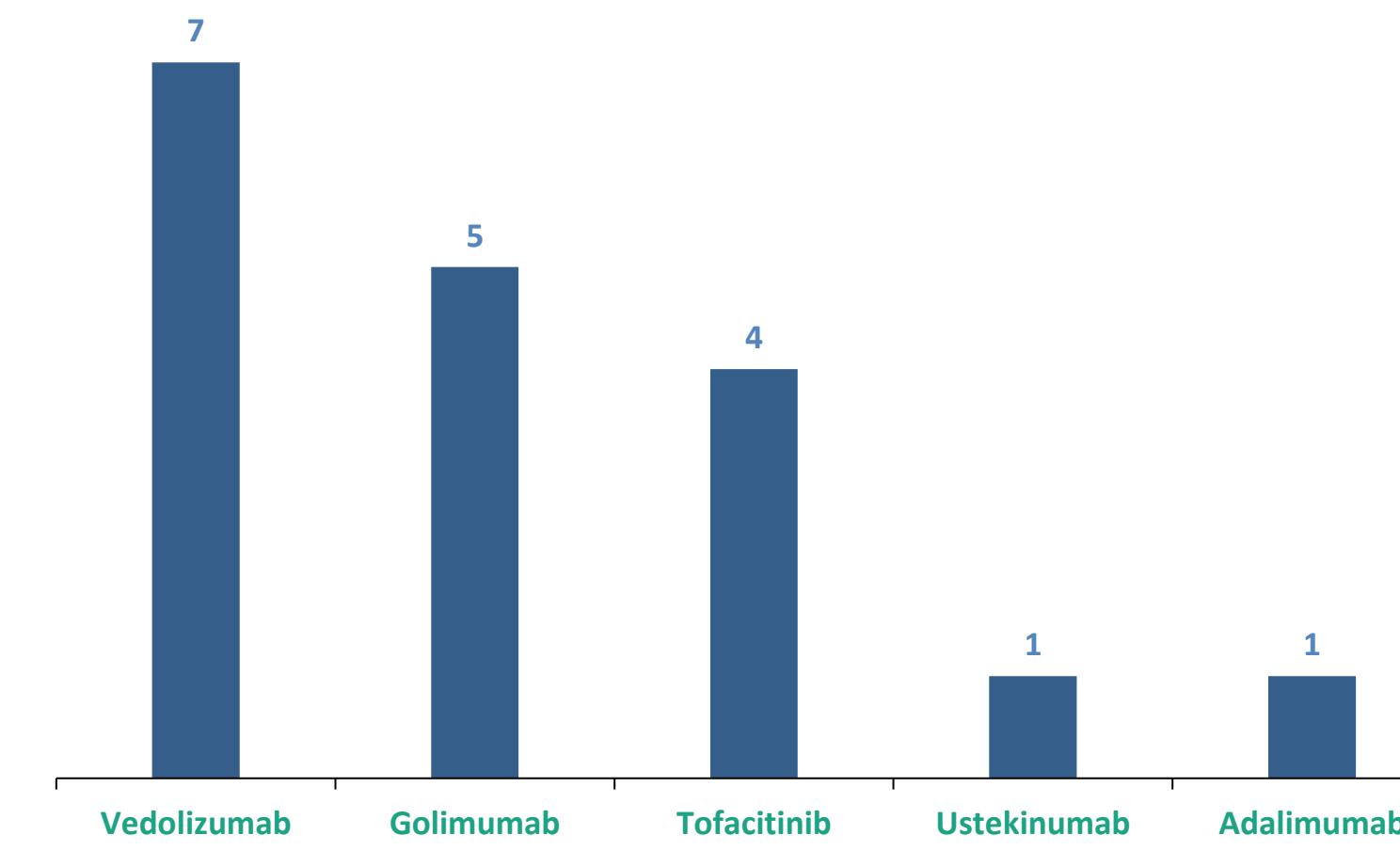


Figure 5. PROMs evaluated across included studies



Abbreviations: EQ-5D: EuroQoL; GSS: Global satisfaction score; HRQoL: Health related quality of life; IBD-DI: Inflammatory bowel disease – disability index; PHQ: Patient health questionnaire; PhGA :Physician global assessment; PROMIS: patient reported outcome measurement information system; PSQI: Pittsburgh sleep quality index; RBS: Rectal bleeding subscore; SF: short form survey; SFS: Stool frequency subscore; SHS: Short health scale; SIBDQ: short inflammatory bowel disease questionnaire; SUS: Stool urgency subscore; VAS: Visual analogue scale; WPAL: Work productivity activity impairment; .

RESULTS

- Of the 4,896 records received from the literature, only 22 studies (<1%) reported PROMs and were included.
- The included studies by publication year are presented in **Figure 1**, indicating a trend of increasing number of publications in recent years.
- Most studies were from Europe (n=13 [59%]) or America (n=6 [27%]). (**Figure 2**). Also, most of the studies were of prospective design (n=17; [77%]).
- Fifteen studies (68%) included a mixed population of biologics-treated and biologics-naïve; 2 studies had a population that was previously treated with biologics, and 5 studies included patients that were not previously treated with biologics (biologics-naïve). (**Figure 3**)
- Most studies focused on a single-treatment (n=18 [82%]), within which vedolizumab (n=7) was the commonly assessed therapy. (**Figure 4**) Additionally, four were comparative studies.
- An overview of PROMs reported across studies is presented in **Figure 5**.
- Fourteen studies (64%) reported at least one disease-specific PROM, comprising IBDQ-32 (n=3; 14%), SIBDQ (n=5; 23%), IBDQ-9 (n=1; 5%), IBD-DI (n=1; 5%), RBS (n=5; 23%), SFS (n=4; 18%), and SUS (n=1; 5%). The commonly used generic PROMs were EQ-5D (23%), SHS (18%), and EQ-VAS (14%). (**Figure 5**)

DISCUSSION

The evidence from this SLR highlights that despite the availability of several disease-specific PROMs in UC disease space, their utilization in real-world clinical practice is very limited. Their low utilization in clinical practice could be explained, in part, to lack of well-defined guidelines on their use in real-world settings, unlike well-defined guidance for their use in clinical trials.

References

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