Assessing Quality-of-Life Outcomes in Ulcerative Colitis: A Review of Canadian HTA Submissions

Brenda Rattanavong[1], Sarah Chami[1], Liga Bennetts[1]

[1]Amaris Consulting, Montreal, Canada

PCR79

INTRODUCTION

- Ulcerative colitis (UC) is a lifelong disease that has considerable impact on patients' quality of life (QoL), affecting many aspects of QoL including psychological, physical, and social domains.[1,2]
- Patient-reported outcome (PRO) and health-related quality-of-life (HRQoL) measures are accepted by regulatory and health technology assessment (HTA) authorities and often complement the clinical evidence of treatments being evaluated.[3,4]
- The process in which HTA agencies evaluate generic and validated disease-specific tools used in submissions for UC therapies is not well characterized.

OBJECTIVE

To understand Canadian HTA bodies' critiques of HRQoL/PRO measures, we reviewed appraisals of outcome measures in HTA submissions in UC.

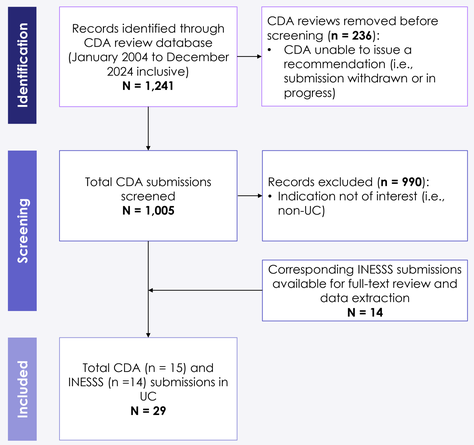
METHODS

- Final recommendation reports of completed HTAs (i.e., not withdrawn or in progress) for UC indications from January 2004–December 2024 inclusive were retrieved from CDA. Corresponding INESSS reports for these products were also retrieved.
- Retrieved CDA and INESSS final recommendation reports were reviewed by two independent investigators to extract the following information:
  - Product under review (e.g., indication, brand and generic name)
  - Submission details (e.g., reimbursement decision)
  - Pivotal trial submitted (e.g., study design, primary endpoint)
  - HRQoL/PRO measure included in submission (e.g., name of measure, generic or disease-specific measure)
  - Appraisal of the measures (e.g., commentary on validity, reliability, and responsiveness of measure, whether minimal clinically important difference (MCID)/minimal important change (MIC) was identified for target population)

RESULTS

- Among 1,005 completed submissions assessed by CDA with a final recommendation issued from January 2004 to December 2024 inclusive, 15 submissions were for 15 UC therapies (**Fig.1**)
  - Fourteen of these UC therapies were also assessed by INESSS during this period.
- PRO/HRQoL measures were mentioned in most CDA and INESSS recommendation reports for UC therapies (22/29 submissions)
  - Across CDA and INESSS recommendation reports for UC therapies, the most frequently mentioned generic PRO/HRQoL instruments include EQ-5D/VAS (n=10 submissions) and SF-36 (n=9; **Fig. 2**)
  - IBDQ/SIBDQ and WPAI-UC were frequently mentioned disease-specific instruments, in 11 and 7 CDA recommendation report, respectively.
    - IBDQ was the only instrument discussed in 14 INESSS final recommendation reports.
  - Eleven submissions to CDA presented both generic and disease-specific instruments.

Figure 1. Overview of identification and selection process for CDA and corresponding INESSS recommendation reports



RESULTS (*cont.*)

Figure 2. PRO/HRQoL measures mentioned in CDA and INESSS recommendation reports for UC therapies

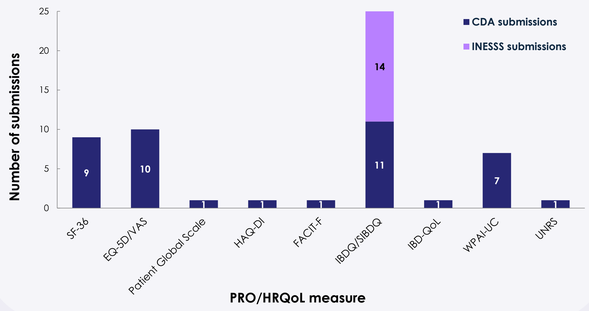


Table 1. CDA appraisal of PRO/HRQoL measurement properties across UC submissions

	PRO/HRQoL Measure (Number of submissions)	Validity of measure is supported?	Measure is reliable?	Measure is responsive?	MCID/MID identified for target population?
Generic measures	SF-36 (N=9)				
	EQ-5D/VAS (N=10)				
	Patient Global Scale (N=1)				
	HAQ-DI (N=1)				
	FACIT-F (N=1)				
Disease-specific Measures	IBDQ/SIBDQ (N=11)				
	IBD-QoL (N=1)				
	WPAI-UC (N=7)				
	UNRS (N=1)				

Note: 'Yes' was the most frequent CDA conclusion for measurement properties that are colored in green. 'No' was the most frequent CDA conclusion for measurement properties that are colored in orange. CDA conclusions were frequently not reported in measurement properties that are colored in grey.

- CDA frequently noted in their appraisal the SF-36 and IBDQ instruments to be valid, reliable, and responsive in five and eight HTAs, respectively (**Table 1**).
  - UNRS was the only disease-specific instrument that CDA identified MCID/MID for UC populations.
  - Whereas for IBDQ, CDA frequently mentioned in submissions (n=8) that an established MCID/MID in patients with UC was not identified.
- INESSS commented on QoL measures in 7/14 reports, focusing on disease-specific measures and their validity. No generic measures were commented on in the INESSS recommendation reports.
- Majority of the submissions in UC (22/29) received a positive reimbursement recommendation from CDA (n=11) and INESSS (n=11).
  - There were no discordant reimbursement decisions between CDA and INESSS.
  - CDA mentioned QoL data in their rationale for recommendation in only eight submissions; in five of these, CDA noted uncertainty in QoL data (e.g., missing patient data, not adjusted for type I error).
    - Among these HTAs with insufficient QoL evidence, one received a negative recommendation.

DISCUSSION & CONCLUSIONS

- Findings show that CDA and INESSS routinely consider QoL data in assessments, with data from clinically-validated instruments and methodologically sound approaches included in appraisals.
  - However, impact of QoL data on decisionmaking is sometimes unclear due to variability in reporting.
- IBDQ was the most frequently included instrument in CDA and INESSS HTA submissions, which was noted to be valid, reliable, and responsive in CDA appraisals.
  - IBDQ has been previously reported in systematic reviews to have the strongest published evidence of validity.[5,6]
  - However, a study by Kim *et al.* evaluated IBDQ using FDA guidance and COSMIN criteria and reported select components (e.g., item and scale refinement, ability to detect change) only partially met FDA guidance and COSMIN criteria.[7]
- Further research is needed to validate MCID/MID thresholds of disease-specific tools in UC populations.

REFERENCES

1. Armuzzi & Liguori. Dig Liver Dis. 2021;53(7):803-808; 2. Benchimol *et al*. J Can Assoc Gastroenterol. 2019; 2(Suppl_1):S1-S5; 3. Doward *et al*. Health Qual Life Outcomes. 2010;8:89; 4. U.S. FDA. Guidance for industry: Patient reported outcome measures: use in medical product development to support labeling claims. 2009 Accessed April 2025; 5. Chen *et al*. Health Qual Life Outcomes. 2017;15(1):177; 6. Alrubaiy *et al*. J Crohns Colitis. 2015;9(3):284-292; 7. Kim *et al*. Qual Life Res. 2024;33(5):1373-1387.

Presented at: ISPOR 2025; 13-16 May 2025; Montreal, QC, Canada

Disclosures: Authors have no conflict of interest to declare.