Integrating Real-world Evidence for Advanced Therapies in Inflammatory Bowel Disease into Reimbursement Submissions: Insights and Considerations from the UK

HTA208

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Background

- Recent studies in inflammatory bowel disease (IBD) highlight notable differences between clinical trial populations and real-world study populations, including age, comorbidities, disease severity, and treatment responses.[1,2] This may lead to limited generalizability of clinical trial results in real-world populations.
- Based on the flexibility of real-world study eligibility criteria, real-world data (RWD) may be able to provide insights into the patient experiences and outcomes of a broader population, which is crucial for making informed decisions about the efficacy and cost-effectiveness of treatments.
- Thus, the UK's National Institute for Health and Care Excellence (NICE) has a strategy to leverage RWD to bridge the knowledge gaps that exist in reimbursement submissions.[3] However, it is not known if RWD was utilized in previous UK NICE health technology assessment (HTA) submissions for advanced IBD therapies.

Objective

- **The objective was to evaluate RWE use in previous NICE HTA submissions for advanced IBD therapies for adults, including biologics and Janus kinase (JAK) inhibitors, and offer recommendations for incorporating real-world evidence (RWE) into future HTA submissions.**

Methods

- A targeted review of NICE appraisals was performed for Crohn's disease (CD) and ulcerative colitis (UC) to understand RWE expectations, integration and usage.
- Available therapies for CD and UC were identified, focusing on those approved for adults. Appraisals for the approved therapies were retrieved from the NICE database. Each appraisal was independently reviewed by two individuals to extract information (year, indication, RWE utilization/context, and RWE study design.)

Results

- As of June 1st, 2025, eight biologics and three JAK inhibitors have been approved for adult CD and UC in Europe.

Figure 1. Approved therapies

	CD	UC
Biologics approved in adults	• Adalimumab • Infliximab • Risankizumab • Ustekinumab • Guselkumab • Mirikizumab • Vedolizumab	• Adalimumab • Golimumab • Infliximab • Risankizumab • Ustekinumab • Guselkumab • Mirikizumab • Vedolizumab
JAK inhibitors approved in adults	• Upadacitinib	• Filgotinib • Tofacitinib • Upadacitinib

Abbreviations: CD = Crohn's disease; IBD = inflammatory bowel diseases; JAK = Janus kinase; UC = ulcerative colitis

Table 1. Evaluation of NICE appraisals for therapies approved for CD and UC in adults from January 2015 to June 2025

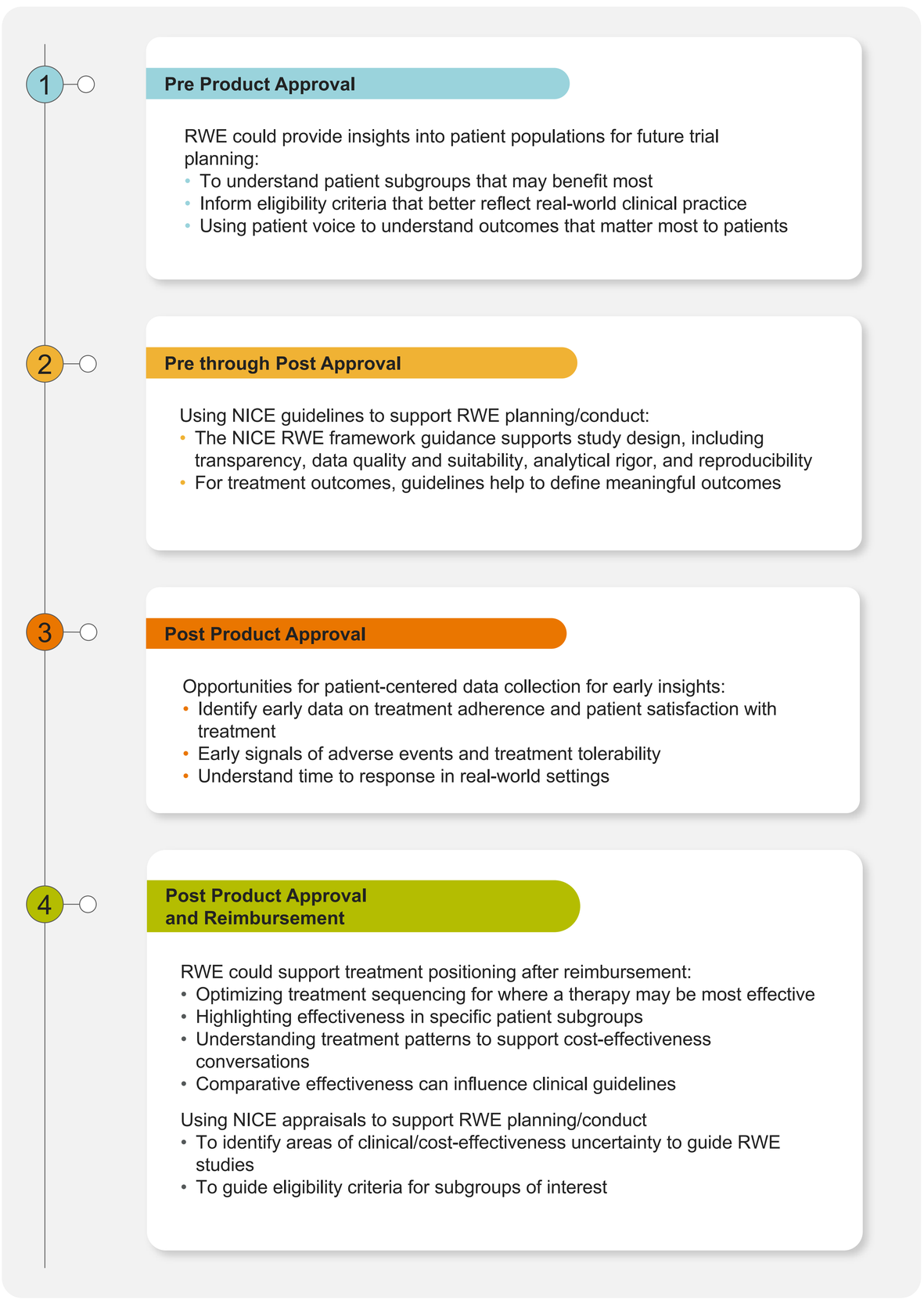
Therapy (appraisal number)	Indication	Year of NICE appraisal	Utilization and context of RWE data
Biologics			
Adalimumab (TA187 [CD] and TA392 [UC]) Appraisal: 2002	For adults with severe active CD, or have not responded to conventional therapies	2002 (Initial); 2010 (updated)	• 2002: No • 2010: Yes, updated appraisal notes additional data on dose escalation from observational studies, used for treatment costs (unknown study design)
	For previously treated moderately to severely active UC	• 2012 (Initial) • 2015 (Additional evidence)	• 2012: No • 2015: Yes, an interim analysis of a prospective study looking at QoL and HRU, to understand HRU rates
Golimumab (TA392)	For previously treated moderately to severely active UC	2015	No
Infliximab (TA187 [CD] and TA392 [UC])	For adults with severe active CD, or have not responded to conventional therapies	• 2002 (Initial) • 2010 (Additional evidence)	• 2002: No • 2010: Yes, updated appraisal notes additional data on dose escalation from observational studies, used for treatment costs (unknown study design)
	For acute exacerbations (2008) For previously treated moderately to severely active UC (2015)	• 2008 (Initial) • 2015 (Additional evidence)	• 2008: No • 2015: No
Risankizumab (TA888 [CD] and TA998 [UC])	For previously treated moderately to severely active CD	2023	No
	For moderately to severely active UC	2024	Yes, this was a cost comparison submission that had RWE informing data for the comparison treatment in cost effectiveness models (unknown study design)
Ustekinumab (TA456 [CD] and TA633 [UC])	For previously treated moderately to severely active	2017	No
	For moderately to severely active UC	2020	No
Guselkumab	For moderately to severely active CD	Has not occurred yet	NA
	For moderately to severely active UC	Has not occurred yet	NA
Mirikizumab (TA925)	For moderately to severely active CD	Has not occurred yet	NA
	For moderately to severely active UC	2023	No
Vedolizumab (TA352 [CD] and TA342 [UC])	For moderately to severely active CD after prior therapy	2015	No
	For moderately to severely active UC	2015	No
JAK inhibitors			
Tofacitinib (TA547)	For moderately to severely active UC	2018	No
Filgotinib (TA792)	For treating moderately to severely active UC	2022	No
Upadacitinib (TA905 [CD] and TA856 [UC])	For previously treated moderately to severely active CD	2023	No
	For treating moderately to severely active UC	2023	No

Abbreviations: CD = Crohn's disease; HRU = health resource utilization JAK = Janus kinase; NA = not applicable; NICE = National Institute for Health and Care Excellence; QoL = quality of life; RWE = real-world evidence; TA = technology appraisal; UC = ulcerative colitis

Results (cont.)

- Sixteen advanced therapy single technology appraisals from 2015–2025 were reviewed.
- RWE was only included in one initial submission, a cost-comparison submission (for a UC therapy).
  - RWE for the comparator informed dose escalation proportions; no RWD used for the treatment appraised.
- RWE was also used in updated submissions for two treatments for CD to help understand treatment costs

Figure 2. Our considerations for integrating RWE into HTA submissions (initial or updated after initial reimbursement)



Abbreviations: NICE = National Institute for Health and Care Excellence; QoL = quality of life; RWE = real-world evidence

Conclusions

- **RWE is under-utilized in HTA submissions to NICE for advanced therapies for IBD.**
- **Future submissions should consider knowledge gaps that could most benefit from RWE integration, including modeling assumptions.**

References

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Disclosures

NRB and **JSS** are employees of PPD™ Observational Studies, Thermo Fisher Scientific. **GM** and **RM** are employees of PPD Evidera™ Health Economics and Market Access, Thermo Fisher Scientific. DC is an employee of PPD™ clinical research business of Thermo Fisher Scientific.

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