HTA52

Early perspectives on health technology assessment in the Kingdom of Saudi Arabia: exploratory survey into market access implications

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Introduction

- In July 2025, the Kingdom of Saudi Arabia (KSA) enacted health technology assessment (HTA) requirements mandating economic evaluations in Saudi Food and Drug Authority (SFDA) submissions.[1]
- This shifts pricing and reimbursement (P&R) decisions from clinical/comparative efficacy to economic value.
- Key operational elements (e.g., willingness-to-pay thresholds, role of international HTA decisions) are still evolving, creating uncertainty overall and for rare diseases.
- This exploratory study describes early stakeholder perspectives on the impact of KSA's evolving HTA.

Objective

To assess stakeholder expectations of KSA's new HTA for P&R/formulary decisions and its implications for rare diseases.

Methods

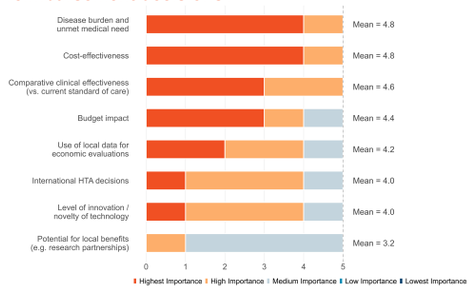
- Five formulary decision makers from the KSA (three national payers, two hospital pharmacists) completed a web-based survey administered via the Rapid Payer Response (RPR™) platform by Genesis Research Group.
- Respondents were asked Likert-style (1=low; 5=high) and open-ended questions to assess early perceptions on the anticipated impact of the KSA's evolving HTA process, including
  - P&R decision drivers under the new HTA
  - Role of international, established HTA outputs (e.g., NICE, CADTH, HAS) in KSA's process
  - Anticipated changes to local decision-making
  - Impact on manufacturers and anticipated challenges
  - Expected special considerations for rare diseases
  - Economic evaluation barriers for rare diseases products
  - Expected effect of the new HTA process on access
- Quantitative responses were summarized using descriptive statistics (counts, percentages). Open-ended responses were described.
- Participation was voluntary and anonymous. No respondent-level data were collected.

Results

Overall

- Respondents identified cost-effectiveness and disease burden/unmet need (mean score = 4.8 for both) as key anticipated drivers of pricing and reimbursement decisions (Figure 1).

Figure 1. Within the new HTA, how important are the following decision drivers for pricing and reimbursement decisions?

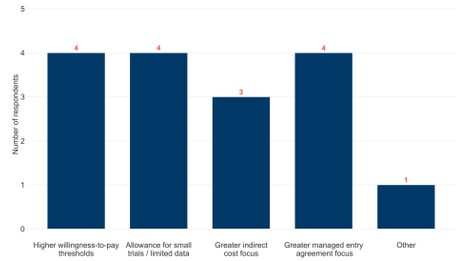


- All respondents (5/5) expected international HTA outputs to be used for benchmarking/validation and methodological guidance. One (1/5) expected use to inform willingness-to-pay thresholds. One (1/5) cited other uses (i.e., informing clinical practice and data requirements).
- Anticipated near-term impact on manufacturers was mixed: 2/5 negative, 2/5 neutral, 1/5 very positive.
- Expected challenges for manufacturers included greater evidence/data requirements (3/5), pricing pressure (3/5), limited access for high-cost products (3/5), longer timelines (3/5), and limited local expertise (1/5).
- Most (3/5) anticipated the new HTA process to shift decisions toward centralized, value-based approaches, with prices linked to cost-effectiveness and budget impact.
  - Several (2/5) expected a phased rollout (initially Ministry of Health/ high-cost technologies); one (1/5) felt private/military/university sectors may retain early flexibility. One (1/5) expected better decision quality and increased formulary inclusion as methods mature. Several anticipated long-term benefits as value rules become clearer and more standardized.

Rare Diseases

- Before the new HTA, respondents described a fragmented approach to rare-diseases:
  - Prices were anchored to external reference pricing with flexibly via case-by-case managed entry and risk-sharing agreements;
  - SFDA incentives (e.g., orphan designation, priority/fast-track review, market exclusivity, scientific consultation) facilitate registration.
- Pre-HTA, three (3/5) noted that high per-patient costs posed a significant barrier to favorable pricing and reimbursement for rare disease products. Two (2/5) cited difficulty demonstrating cost-effectiveness. None selected limited clinical trial data / sample size or lack of local epidemiology data / other data as a significant barrier.
- Respondents anticipated rare disease-specific allowances under the new HTA (Figure 2).

Figure 2. Which special considerations do you expect will be given to rare disease therapies being reviewed under the new HTA system?



- For rare diseases, three (3/5) respondents anticipated improved access after the HTA (Figure 3), though one caveated that this only applies to therapies that can demonstrate cost-effectiveness; access may be restricted for others.

Figure 3. Do you anticipate the new HTA process to promote or restrict patient access to new rare disease therapies?

Limitations

- This study used a small, purposive sample (n=5) in an early-stage policy environment.
- Views reflect anticipated near-term effects.

Conclusions

- **The KSA's new HTA process is expected to shift decision-making toward a more centralized, transparent, and value-based framework.**
- **International HTA decisions will likely serve as a methodological reference, but local evidence is expected to remain important for decision-making.**
- **For manufacturers, the new HTA process may increase evidence requirements, pricing pressure, and overall market access complexity.**
- **For rare diseases, some flexibility is anticipated in evidentiary and decision thresholds, supported by greater use of managed entry agreements.**
- **Overall, the anticipated effect on rare disease access is cautiously optimistic, with the ultimate impact likely to depend on how flexibly the framework is implemented in practice.**

Disclosures

This study was conducted and funded by Genesis Research Group. Amie Devlin, Darrin Benjumea, and Tijana Ignjatovic are employed by Genesis Research. Lynn Okamoto was employed by Genesis Research Group at the time of this study.

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The False Confidence of Unanchored MAICs: Lessons in Ulcerative Colitis

MSR103

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Introduction

- Indirect comparisons are used in the absence of RCTs.
- Matching-adjusted indirect comparison (MAIC) helps compare effectiveness of treatments when individual patient data (IPD) are available for one trial and published summaries are available for another.
  - **Anchored MAICs** compare treatment effects between studies with a shared common comparator, such as placebo
  - **Unanchored MAICs** do not have such an anchor.
- The catch is that unanchored MAICs, despite being less robust and using less information, often yield:
  - Narrower confidence intervals
  - Smaller p-values

Objective

- **To compare anchored and unanchored MAICs for ustekinumab versus published ulcerative colitis therapies during induction, focusing on statistical significance and the width of 95% confidence intervals.**

Methods

- UC studies are well-suited to this task:
  - Similar placebo-controlled designs
  - Similar eligibility criteria
  - Closely aligned endpoints
- IPD from the UNIFI phase III trial of ustekinumab in moderately to severely active ulcerative colitis (UC), obtained through Yale University Open Data Access Project.

Comparator treatments

- vedolizumab, upadacitinib, mirikizumab, guselkumab, etrasimod, and adalimumab

Outcomes

- Clinical response & clinical remission to induction therapy

Statistical analyses

- Each comparison was performed twice:
  - Anchored: preserved the shared placebo comparator
  - Unanchored: intentionally omitted placebo
- IPD from UNIFI was weighted to match baseline characteristics of each of the comparator trials.
- Results reported on the risk difference and odds ratio scales

Results

Table 1: Baseline Characteristics for UNIFI

Characteristic	Ustekinumab (6 mg/kg) N = 322	Placebo N = 319
Age	41.7 (13.7)	41.2 (13.5)
Male Sex	195 (61%)	197 (62%)
White Race	243 (76%)	248 (78%)
BMI	24.7 (5.4)	24.5 (4.8)
Left-sided disease	169 (53%)	168 (53%)
Mayo Score	9.0 (1.5)	8.9 (1.6)
Endoscopic subscore = 3	240 (75%)	216 (68%)
Prior Anti-TNF therapy	166 (52%)	175 (54%)
Duration of UC (years)	8.5 (8.0)	8.3 (7.4)
Fecal calprotectin (mg/kg) – Median (IQR)	1586 (740, 3176)	1403 (582, 2563)
CRP (mg/L) – Median (IQR)	5.2 (1.8, 13.7)	5.0 (1.4, 10.7)
Concomitant glucocorticoid use	179 (56%)	161 (51%)
Concomitant ASA use	240 (75%)	207 (65%)
Concomitant immunomodulator use	94 (29%)	90 (28%)

ASA = aminosalicylates; BMI = body-mass index; CRP = C-reactive protein; IQR = interquartile range; TNF = tumor necrosis factor; UC = ulcerative colitis.

Continuous variables are presented with mean and standard deviation unless otherwise specified.

Minimal missingness was handled using a single random-forest imputation; values may differ slightly from published Table 1.

Figure 1 – Results for RD with and without anchoring

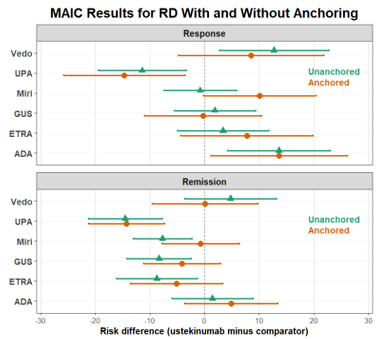
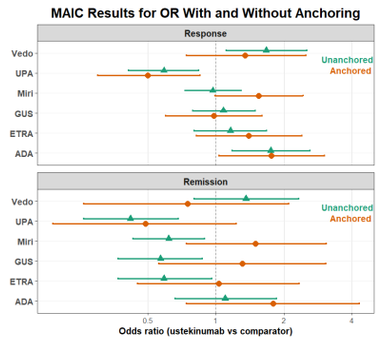


Table 2: Raw Outcomes for Each Trial

Comparator	Active %	Placebo %	Naïve Unanchored RD	Naïve Anchored RD
Clinical response to induction therapy (USTE 61.8%; placebo 31.3%)				
Vedolizumab	47.1%	25.5%	14.7%	8.8%
Upadacitinib	73.6%	26.2%	-11.8%	-17.0%
Mirikizumab	63.5%	42.2%	-1.7%	9.2%
Guselkumab	61.5%	27.9%	0.3%	-3.2%
Etrasimod	62.3%	37.2%	-0.5%	5.4%
Adalimumab	50.4%	34.6%	11.4%	14.6%
Clinical remission to induction therapy (USTE 15.5%; placebo 5.3%)				
Vedolizumab	16.9%	5.4%	-1.4%	-1.3%
Upadacitinib	29.8%	4.3%	-14.3%	-15.4%
Mirikizumab	24.2%	13.3%	-8.7%	-0.7%
Guselkumab	22.6%	7.9%	-7.0%	-4.5%
Etrasimod	26.0%	10.9%	-10.5%	-4.9%
Adalimumab	16.5%	9.3%	-1.0%	3.0%

Figure 2 – Results for OR with and without anchoring



- Mean CI width was smaller for **unanchored** vs **anchored** MAICs on both scales:
  - **RD:** 15.2 vs 19.6
  - **log(OR):** 0.82 vs 1.40

Conclusions

- **Unanchored MAICs produce narrower confidence intervals and smaller p-values than their anchored counterparts even though they use less data and rely on stronger assumptions.**
- **Additional caution should be taken when interpreting unanchored MAICs, recognizing these limitations and the increased chance for bias.**
- **Researchers and analysts should never deliberately use an unanchored MAIC when an anchored one is feasible merely to gain precision.**

Disclosures

Bret Zeldow completed this work while employed by Genesis Research Group, not in connection with his current affiliation. Craig Parzynski is an employee of Genesis Research Group.

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