Value of Multi-Indication Immunosuppressive Therapies (ISTs) for the Treatment of Autoimmune Diseases in the United States (US)

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Background

- Drugs for autoimmune diseases are often approved for multiple indications, for which their cost-effectiveness varies.[1-3]
- Compared to single-indication launches, multi-indication assets face greater strategic and operational complexity (eg, indication sequencing, copositioning, pricing, performance).[4]
  - Determining the best order to launch various indications is a crucial initial strategic decision for a multi-indication asset. Manufacturers must choose between 2 primary sequencing strategies: "narrow first" (highest benefit) or "broad first" (high number of patients impacted).
- Due to the single-price reimbursement model, United States (US) healthcare payers struggle with aligning a single price to each drug's differing value.[5]
- Given the significance of these variables on access and reimbursement, it is important to explore different pricing models in the US that can better align clinical benefit and price to ensure launch success.

Objective

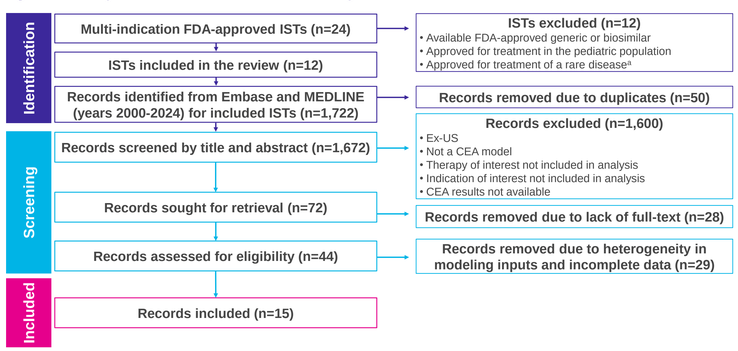
- To examine the clinical and economic value, price, and launch strategies of multi-indication ISTs for the treatment of autoimmune diseases in the US.

Methods

This study was conducted in 5 steps:

1. Selection of multi-indication ISTs through database review (**Figure 1**)
   - Review of approved product portfolios using Biomedtracker and Datamonitor (up to July 2024)
   - The selected multi-indication ISTs served as a batch representation for all treatments available for autoimmune diseases
2. To measure sequencing strategy for multi-indication launches, a review of disease prevalence data for each indication was performed
   - Prevalence data were obtained from an ad-hoc search of US patient advocacy group webpages
3. To measure the clinical and economic benefit each indication offers, a literature review of economic evaluations across Embase and MEDLINE (November 2000 to November 2024) was performed (**Figure 1**)
   - Data on total quality-adjusted life-years (QALYs) derived from cost-effectiveness analyses (CEAs) within the context of US healthcare were sourced from peer-reviewed literature
   - Two reviewers worked independently to perform the screening and data extraction using DistillerSR. Any discrepancies between the 2 reviewers were resolved through discussion
4. To measure changes in list price over time, an analysis of wholesale acquisition cost (WAC) unit pricing history was performed
   - WAC was obtained from Medi-Span® Price Rx Pro® Plus (up to January 2025)
5. Analysis of WAC unit pricing history, prevalence, and CEA data of included ISTs
   - WAC unit pricing history was evaluated as changes over time mapped over the launch date of each indication
   - Total QALY data for each subsequent indication launch was compared against the first indication using the Mann-Whitney U test
   - Prevalence data was pooled for each indication launch sequence and represented as median and range

Figure 1. Targeted literature review flow diagram



[a] Rare disease is defined as a disease or condition that affects less than 200,000 individuals in the US.
Key: CEA – cost-effectiveness analysis; FDA – Food and Drug Administration; IST – immunosuppressive therapy; US – United States.

Results

Data pool

- Twelve multi-indication ISTs, with no available approved generic or biosimilar, across 10 indications were identified and included in the targeted literature review.
- Due to heterogeneity of modeling inputs, availability of CEA data, and accessibility to full-text articles, 5 multi-indication ISTs were included in the final analysis (**Table 1**).
  - Data on QALYs were only published for a select subset of indications.

Table 1. Sample of multi-indication ISTs included in the final analysis

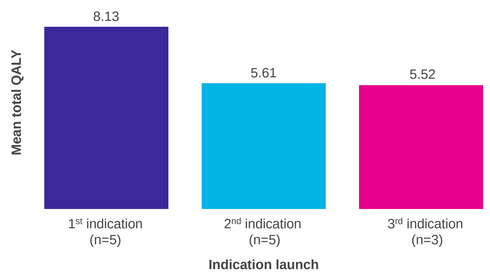
Drug	Number of indications[a]	1st FDA approval
Golimumab	4	June 27, 2008
Secukimumab	6	January 21, 2015
Upadacitinib	7	August 16, 2019
Certolizumab pegol	7	April 22, 2008
Vedolizumab	2	May 20, 2014

[a] The number of indications include FDA-approved indications for both adult and pediatric populations. This analysis is focused solely on FDA-approved indications for the adult population.
Key: CEA – cost-effectiveness analysis; FDA – Food and Drug Administration; IST – immunosuppressive therapy; US – United States.

Mean total QALY

- The first approved indications for ISTs provided numerically higher clinical benefits as measured by mean total QALYs (8.13; 95% confidence interval [CI]: −1.00, 17.27) compared to the second indications (5.61; 95% CI: 2.52, 8.70; *P*=0.62), and third indications (5.52; 95% CI: 5.31, 5.74; *P*=0.76) (**Figure 2**).
- Average mean total QALYs decreased with the launch of each subsequent new indication (**Figure 2**).
- Data on mean total QALYs were only reported for a subset of indications due to lack of available publications on economic models.

Figure 2. Mean total QALY by indication launch sequence

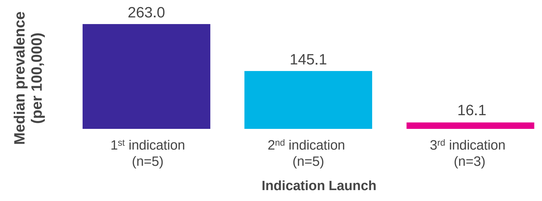


Key: QALY – quality-adjusted life-year.

Median disease prevalence

- The median US disease prevalence per 100,000 individuals was 263.0 (range: 63.8 to 739.0) for first approved indications compared to 145.1 (range: 6.0 to 739.0) for second approved indications, and 16.1 (95% CI: 6.0 to 6,824.0) for third approved indications (**Figure 3**).

Figure 3. Median US disease prevalence by indication launch sequence



Key: US – United States.

Indication launch decisions

- Under a single-price policy, theory suggests that manufacturers may be incentivized to sequence and withhold indications according to the clinical value and number of patients to extract the highest possible price (**Figure 4a**).[6]
- Evidence from this study suggests ISTs are first launched for autoimmune diseases that offer the highest QALY in a larger patient population and then extended to indications that deliver lower QALY to smaller patient populations (**Figure 4b**).

Figure 4. Relationship between mean total QALY and disease prevalence for indication launches

Figure 4a. Indication launch sequencing theory

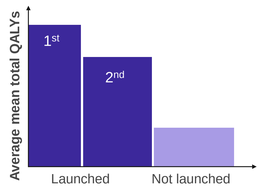
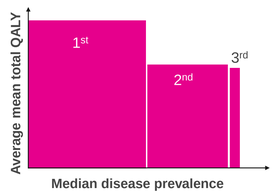


Figure 4b. Indication launch sample evidence

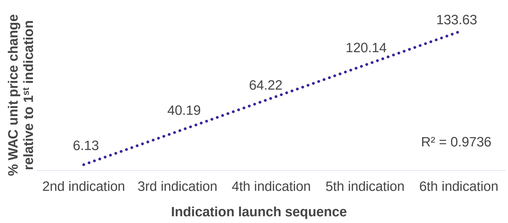


Key: QALY – quality-adjusted life-year.

WAC history

- The average total WAC percent change increased with each subsequent indication launch compared to the first indication launch as suggested by the correlation coefficient (R^2=0.9736) (**Figure 5**).
  - The average percent change ranged from 6.13% (second indication launch) to 133.63% (sixth indication launch).

Figure 5. WAC history by indication launch sequence



Key: WAC – wholesale acquisition cost.

Limitations

- Due to the lack of a formal health technology assessment process in the US, economic data were retrieved from peer-reviewed publications. There was a lack of published data for all approved indications for each immunotherapy; therefore, sample size was limited.
- Risk of bias was not determined for the available evidence. The heterogeneity between modeling inputs for all the CEAs will result in variation for the true effect sizes; therefore, results may be inconsistent and may not be pooled.
- The presence of combination therapies may influence the results as some ISTs are considered add-on treatment for its approved indication; therefore, the calculated clinical value is not solely represented by the immunotherapy.
- Treatment sequences vary for all the ISTs. For example, some treatments are considered second- or third-line for its approved indication; therefore, clinical value might vary between each subsequent indication.
- The results of this study may not be generalizable to all ISTs approved for the treatment of autoimmune disease. This study reflects the available data for a select few drugs.

Conclusions

- Within the autoimmune disease space, the average clinical benefits of ISTs and disease prevalence decrease with each subsequent indication launch, while the average total WAC percent change increases.
- Evidence suggests manufacturers tend to prioritize launching drugs with the highest clinical value and largest disease prevalence, especially in the case of autoimmune diseases, thus supporting the theory that a favorable price is set for the initial indication that reflects superior outcomes. As additional indications are launched, this initial price provides a favorable reference point for any price negotiations of future indications.
- Evidence from this study highlights the misalignment between clinical value and price, under the current single-price reimbursement model, for multi-indication assets.
- As more manufacturers are prioritizing multi-indication assets, further research is required to explore the need for different pricing models in the US that can better align clinical benefit and price to ensure commercial success.

Acknowledgments

- The authors would like to acknowledge Rebecca Vann for providing graphical support; Jessica Beifuss, ELS, for conducting quality review; and Karen Rascati, PhD for providing strategic support.

References

1. Mauskopf J, et al. *Pharmacoeconomics*. 2014;32(4):395-409. **2.** Jung SM, et al. *Immune Netw*. 2022;22(1):e9. **3.** Ha SY, et al. *Int J Environ Res Public Health*. 2022;19(7):4105. **4.** IQVIA. Success multiplied: launch excellence for multi-indication assets. February 2023. Accessed March 15, 2025. https://www.iqvia.com/-/media/iqvia/pdfs/library/white-papers/iqvia-launch-excellence-for-multi-indication-assets-02-23-forweb.pdf **5.** Institute for Clinical And Economic Review. Indication-specific pricing of pharmaceuticals in the United States health care system. March 2016. Accessed March 15, 2025. https://icer.org/wp-content/uploads/2021/01/Final-Report-2015-ICER-Policy-Summit-on-Indication-specific-Pricing-March-2016_revised-icons-002.pdf **6.** Michaeli DT, et al. *Appl Health Econ Health Policy*. 2022;20(5):757-768.

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Presented at: ISPOR 2025
May 13-16, 2025; Montreal, Quebec, Canada
Funded by Cencora

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