Payer perceptions of ICER's impact on rare disease assessment

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Background

- The Institute for Clinical and Economic Review (ICER) value assessment framework (VAF) has increasingly influenced payer decision making in recent years
- A 2020 study conducted by Xcenda found that 72% of payers surveyed stated that ICER recommendations influenced their coverage decisions, which increased from 49% in 2016[1]
- There is limited evidence assessing ICER's influence on payer decision making in therapies for rare diseases

Objective

- To understand the impact of ICER reports in rare diseases in context with other therapeutic areas (TAs) and to analyze trends in ICER's findings for therapies for rare diseases

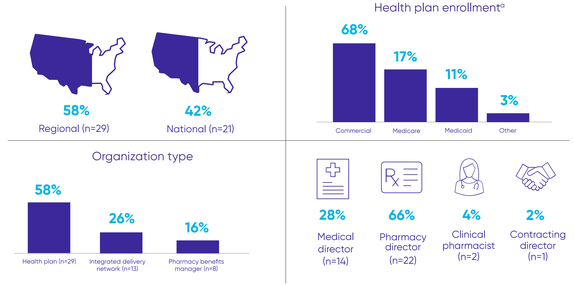
Methods

- A double-blinded web-based payer survey containing multiple choice and open-ended questions was fielded to Xcenda's Managed Care Network (MCN) from September 28, 2021, through October 10, 2021
  - MCN is a proprietary research panel of healthcare executives, medical and pharmacy directors, and other experienced individuals in managed care
  - Participation in this survey was voluntary, and a modest honorarium was paid by Xcenda to participants who completed the survey
  - The survey focused on ICER-related topics, including the level of impact ICER assessments have on an organization's decision-making process based on TAs and reasons for why an ICER assessment might be most impactful
- ICER reports from 2017 to 2021 were analyzed to identify assessments in rare diseases, which are defined by ICER as a patient population of fewer than approximately 200,000 individuals in the United States (US)
  - A total of 12 rare disease assessments were identified in the following indications: multiple sclerosis, primary progressive and secondary progressive retinal disease, chimeric antigen T-cell receptor (CAR-T) pediatric B-cell acute lymphoblastic leukemia (B-ALL), cystic fibrosis, amyloidosis, hereditary angioedema, spinal muscular atrophy, Duchenne muscular dystrophy, sickle cell disease, hemophilia A, and lupus nephritis
  - Overall, 34 therapies were identified in the rare disease assessments, and ICER findings on cost per quality-adjusted life-year (QALY), wholesale acquisition cost (WAC) discount rates, and value-based price benchmarks for willingness-to-pay (WTP) thresholds of $100,000/QALY and $150,000/QALY were summarized
    - Details on these findings were extracted from the ICER reports for each therapy

Results

- There were a total of 50 payers who completed the survey. As shown in **Figure 1**, health plans represented the largest percentage of respondents' organizations, followed by integrated delivery networks.

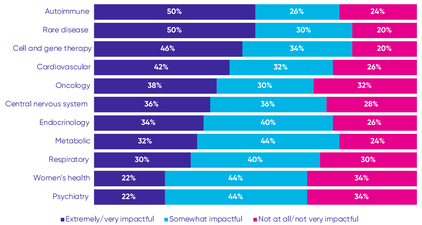
Figure 1. Respondent demographics (N=50)



[a] Health plan enrollment estimate is indicated by number of covered lives served for each line of business and excludes duplicate health plans and pharmacy benefit managers.

- Approximately half of respondents (46%–50%) claimed ICER assessments are extremely or very impactful, and over 75% thought ICER assessments are at least somewhat impactful on an organization's decision-making process in rare diseases, autoimmune diseases, and cell and gene therapies (**Figure 2**)

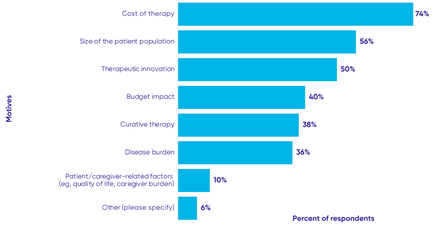
Figure 2. Impact of ICER on the healthcare decision-making process by TA (N=50)



Q: Please rate the level of impact an ICER assessment would have in your organization's decision-making process in the following TAs.

- Over half of respondents (56%–74%) indicated that the cost of therapy followed by the size of the patient population are primary motives for ICER's impact variance among TAs (**Figure 3**)

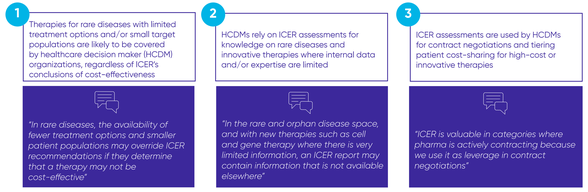
Figure 3. Motives for ICER impact variance among therapeutic areas (N=50)



Q: Why might an ICER assessment be more impactful to you for one TA over another?

- The top 3 reasons for utilization of ICER reports in rare diseases by payer respondents are summarized in **Figure 4**

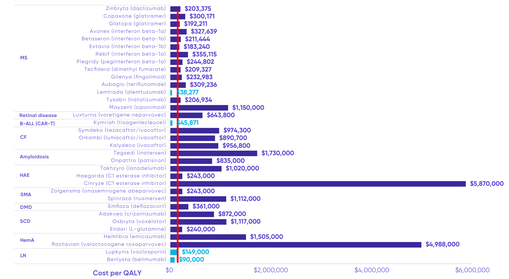
Figure 4. Top 3 payer reasons for utilization of ICER reports in rare diseases



Q: Please describe your reasoning.

- An analysis of ICER reports from 2017 to 2021 in therapies for rare diseases showed that ICER found only 4 of 34 interventions to be cost-effective at a WTP of $150,000/QALY (**Figure 5**)

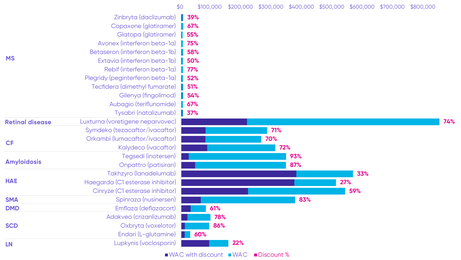
Figure 5. Cost per QALY of ICER-assessed rare disease therapies



Note: Blue font = Cost-effective at a WTP threshold of $150,000/QALY.
Key: B-ALL = B-cell acute lymphoblastic leukemia; CAR-T = chimeric antigen T-cell receptor; CF = cystic fibrosis; DMD = Duchenne muscular dystrophy; HAE = hereditary angioedema; HemA = hemophilia A; LN = lupus nephritis; MS = multiple sclerosis; SCD = sickle cell disease; SMA = spinal muscular atrophy.

- ICER value-based price benchmarks suggested the need for WAC discounts ranging from 27% to 98% to reach a WTP of $100,000/QALY and 22% to 93% to reach a WTP of $150,000/QALY (**Figure 6**)

Figure 6. ICER-estimated discounts from WAC to reach WTP of $150,000/QALY



Note: Only therapies that required a WAC discount to reach a WTP of $150,000 are presented in the chart. Mayzent (siponimod), Zolgensma (onasemnogene abeparvovec), Hemlibra (emicizumab), Roctavian (valoctocogene roxaparvovec), and Benlysta (belimumab) did not have specified discounts. Kymriah (tisagenlecleucel) was noted by ICER to need an increase of WAC by 255% and Lemtrada (alemtuzumab) an increase of WAC by 390%.
Key: CF = cystic fibrosis; DMD = Duchenne muscular dystrophy; HAE = hereditary angioedema; LN = lupus nephritis; MS = multiple sclerosis; SCD = sickle cell disease; SMA = spinal muscular atrophy.

Study limitations

- Survey results were descriptive in nature and based on a small number of respondents and thus may not be generalizable to all payer organizations or payer types
- Because all respondents voluntarily completed the survey, voluntary response bias may exist, and survey results may over-represent respondents with stronger interest in payer-manufacturer partnerships
- This research reflects the perspectives of managed care professionals identified from Xcenda's MCN research panel; other user types (eg, healthcare providers, patients, manufacturers) were not represented in this subset

Conclusions

- While payers identify ICER's reports in therapies for rare diseases to be highly impactful, these reports nearly always conclude that treatments for rare diseases are not cost-effective and do not seem to affect coverage decisions for these treatments
- Still, payers noted ICER assessments of therapies for rare diseases and cell and gene therapies to be particularly valuable and may be considered in contract negotiations and when tiering patient cost-sharing, since there is generally limited information and/or internal data/expertise in rare diseases
- Future research among payers will be critical to understand whether higher cost-effectiveness thresholds are warranted for rare diseases in the evolving value-based landscape

Reference

1. Choi M, Hydery T, Tan R, Tennant L. Trends in the uptake and impact of the Institute for Clinical and Economic Review value assessment framework in payer coverage decisions from 2016 to 2020. Presented at: 2021 AMCP Virtual Meeting April 12–16, 2021.

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