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What utilization management strategies does ICER recommend to US payers and policymakers to manage uncertainty with gene therapies?

Rita Conceição, Priyanka Narvekar, Peter Okorozo, Jennifer Sander; Avalere Health, USA

Introduction

- The Institute for Clinical and Economic Review (ICER) assesses the value of healthcare interventions to inform pricing and coverage decisions by US payers.
- In addition to the assessment of value, ICER routinely publishes recommendations for payers and policymakers; however, no guidelines exist for the development of these recommendations.
- Understanding ICER's recommendations would help pharmaceutical companies to price their products competitively within the ICER framework. Furthermore, the recommendations from ICER can provide context around how US payers might respond to novel therapies, including gene therapies, that carry significant uncertainties regarding their long-term effectiveness, thereby enabling pharmaceutical companies to anticipate challenges or opportunities for their novel drugs.
- US payers tend to manage uncertainty in the evidence supporting an intervention by implementing management utilization strategies such as prior authorizations (PA), step therapy (ST), or quantity limits.

Objective

- To understand utilization management strategies recommended by ICER to US payers when areas of uncertainty are raised in ICER assessments of gene therapies.

Methods

- We reviewed ICER's policy recommendations for the following gene therapies: Luxturna, Zolgensma®, and Spinraza® (joint assessment for spinal muscularatrophy [SMA]) and Hemgenix for hemophilia.
- We identified the areas of uncertainty highlighted in the assessment, considering key words such as "uncertainty," "remaining uncertainty," "lack of," "long-term treatment effect," "long-term safety and efficacy data," and "cost-effectiveness thresholds."
- The uncertainties highlighted in the assessments were linked with the recommendations provided to payers and policymakers.

Results

- In the assessment of Hemgenix, ICER recognized there was no approved treatment (except prophylaxis) for hemophilia and recommended that payers should not use ST as a utilization management strategy. Hemgenix was considered cost-effective compared with current prophylaxis based on lifelong cost offset data (Table 1), despite the high price and lack of long-term efficacy data.[1]
- In the SMA assessment, ICER noted the new mechanism of action, lack of long-term safety and efficacy data, and high price of Spinraza® and Zolgensma® as limiting factors and recommended that payers implement outcomes-based contracts OBCs. As a result of the high price of Zolgensma® and existence of Spinraza® in the market, ICER also recommended that payers develop PA criteria (Table 1).[2]
- Overall, if a standard-of-care treatment is not available, ICER recommends the use of PA. If a standard-of-care therapy is available, PA in combination with ST is recommended instead.
- If the implementation of OBCs is feasible, ICER highlights this as a possibility to address uncertainty in long-term treatment effect.

Table 1: ICER's key recommendations on policy considerations for gene therapies

Key drivers for policy recommendations	Key ICER recommendations for payers
Uncertainty in long-term treatment effect	Payers should work with drug manufacturers to implement outcomes-based agreements in order to address uncertainty in the treatment effect and the high costs of the gene therapies considered in this research.
Lack of standard-of-care therapy precludes ST as a utilization management strategy	ST is not applicable to Hemgenix. Clinical and patient experts view this approach as lacking any clinical justification and consider it to be a method for avoiding the high one-time fee for gene therapy while assuming that patients may switch insurers before the cost-saving potential of gene therapy is fully realized.[1]
Implementation of management utilization through PA due to high cost	Given the substantial uncertainty in the benefit of Spinraza® and Zolgensma® in certain patient subgroups and the high cost of these therapies, ICER recommended the use of PA.[2] PA criteria should be clear for providers and should be based on evidence presented in clinical guidelines.

> "Given that Spinraza and Zolgensma have new mechanisms of action, lack long-term safety and efficacy data, and are very expensive, it is reasonable for insurers and other payers to negotiate outcomes-based contracts with manufacturers."

> "Given the substantial remaining uncertainty regarding the benefits of these treatments in certain subpopulations and their high cost, it is reasonable for insurers and other payers to develop prior authorization criteria to ensure prudent use."

Conclusions

- Although there are no guidelines for ICER's payer and policy recommendations, for gene therapies, these seem to be driven by high costs and uncertain long-term treatment effects.
- ICER recommends PA, ST (alone or in combination), and OBCs as strategies for US payers and policymakers to manage uncertainty when deciding on the coverage of gene therapies.
- ICER's policy roundtables recommend that payers explore innovative approaches to coverage and pricing in order to manage uncertainty and high costs without restricting access.

References

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2. ICER. Spinraza® and Zolgensma® Final Report. 2019. https://icer.org/news-insights/press-releases/icer-issues-final-report-on-sma/

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