Understanding the Challenges of Evaluating and Managing Orphan Drugs from the Payer, Provider, and Employer Perspectives

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Background

- As an increasing number of orphan drugs are FDA approved, healthcare payers, employers, and providers are attempting to strike a balance between patient access to innovative treatments and overall affordability
- Payers and providers are considering additional types of clinical and economic data to understand the overall value of high-cost drugs for rare diseases

Objective

To describe the challenges that healthcare stakeholders are facing in managing orphan drugs, and how payers and providers are evaluating orphan drugs.

Methods

- A survey was conducted with payer, provider, and employer decision-makers recruited from both AMCP and a proprietary database of market-access decision-makers
- Survey was fielded using Qualtrics platform between July 16, 2020 and August 4, 2020
- The survey consisted of a total of 35 questions, with branching logic to direct segment-specific questions to payers, providers, and employers
- Respondents were asked about their experiences and activities in the orphan disease space
- The survey was double-blinded and participation was voluntary. Survey respondents received an honorarium for participation

Results

Respondents: Payers n = 26; Providers n = 21; Employers n = 11

Figure 1: **How great a challenge is each of the following in your organization?**

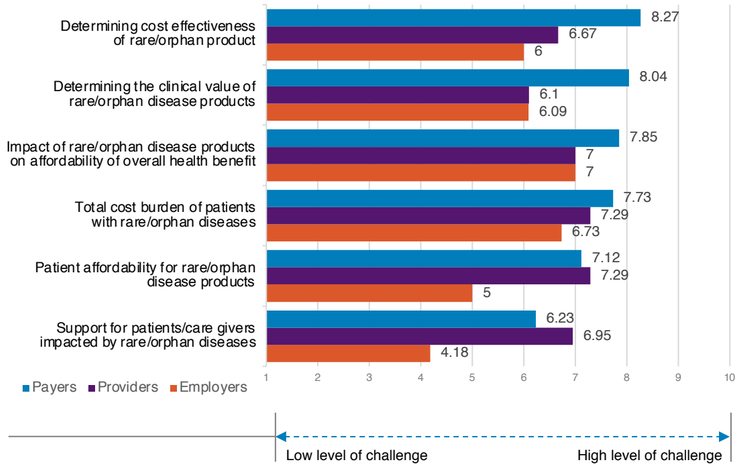


Figure 2: **How challenging are each of the following issues with regard to clinical evaluation of rare/orphan disease drugs in your organization? (Payer and provider respondents)**

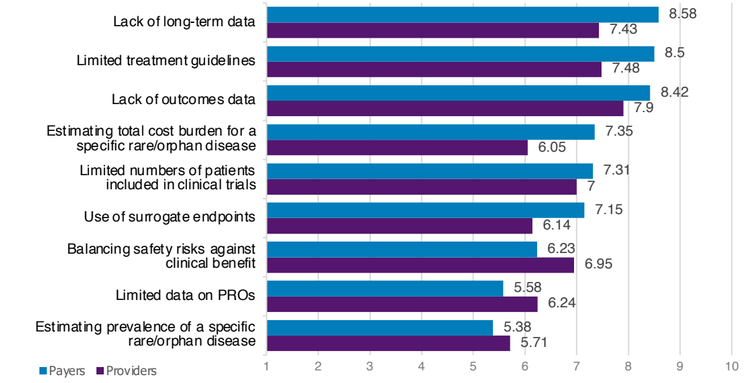


Figure 3: **Does your organization currently use or plan to use any of the following to drive decision-making for rare/orphan disease drugs? (Payer respondents)**

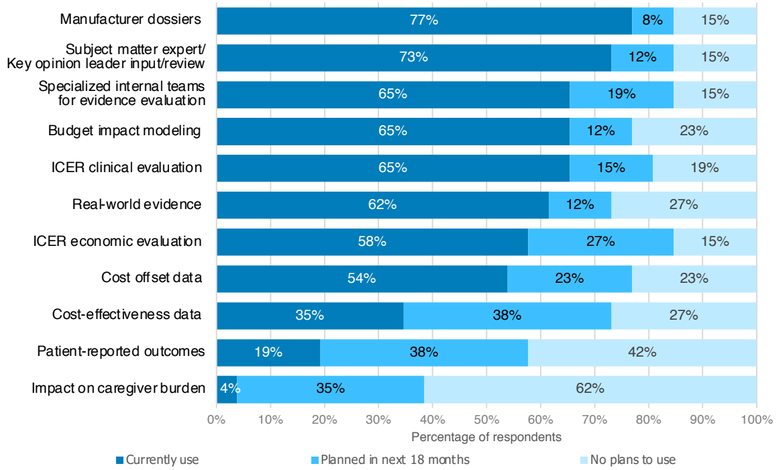
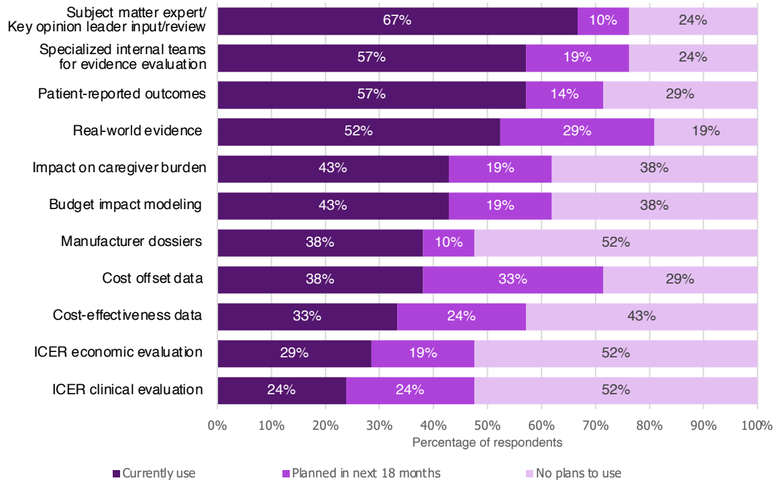


Figure 4: **Does your organization currently use or plan to use any of the following to drive decision-making for rare/orphan disease drugs? (Provider respondents)**



Conclusions

- Healthcare stakeholders are faced with multiple challenges in managing orphan drugs, particularly in overall affordability, and in determining the cost-effectiveness and clinical value of these products
- Payers and providers are increasingly incorporating healthcare economic data and patient-reported outcomes into evaluation of orphan drugs

Limitations

- Small sample size did not allow for comparisons between segments
- Participation bias may have driven individuals with more interest or knowledge to respond to the survey
- The survey design included primarily closed-ended questions with pre-populated responses; findings may not have captured emerging or infrequently encountered trends

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