Understanding Payer, Provider, and Employer Resources to Support Patients and Caregivers Impacted by Rare Diseases

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Background

As an increasing number of orphan drugs are FDA approved, healthcare payers, employers and providers are considering opportunities to better support these complex patients and their caregivers who are impacted by rare diseases.

Objective

To understand current and future strategies to optimize the care experience for patients with rare disease, including use of patient reported outcomes (PROs).

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	Providers	Employers
	n = 26	n = 21	n = 11

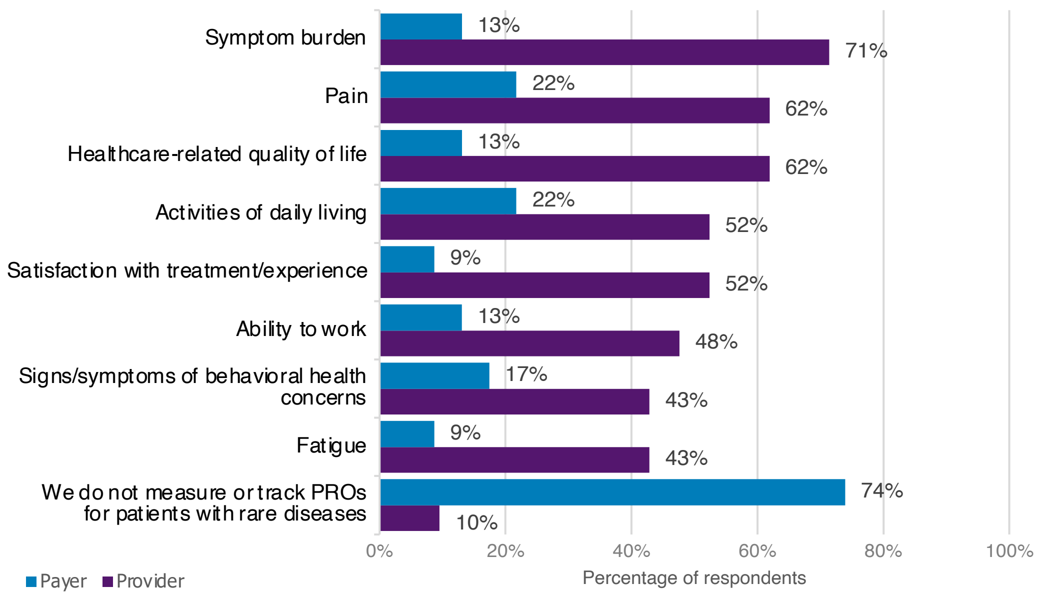


Figure 1: **Do you measure or track any of the following PROs for your patients with rare/orphan diseases? (Payer and provider respondents only)**

Table 1: **How does your organization track PROs? (Payer and provider respondents only)**

Method of tracking PROs	Payers (n=6)	Providers (n=19)
Surveys, interviews, diaries	2	3
Leverage specialty pharmacy	2	2
Electronic medical records/patient charts	1	3
Online portal	0	1
Registries	0	1
Nurse follow-up	0	1

Figure 2: **What resources/programs does your organization provide for patients with rare/orphan diseases? (Payer respondents)**

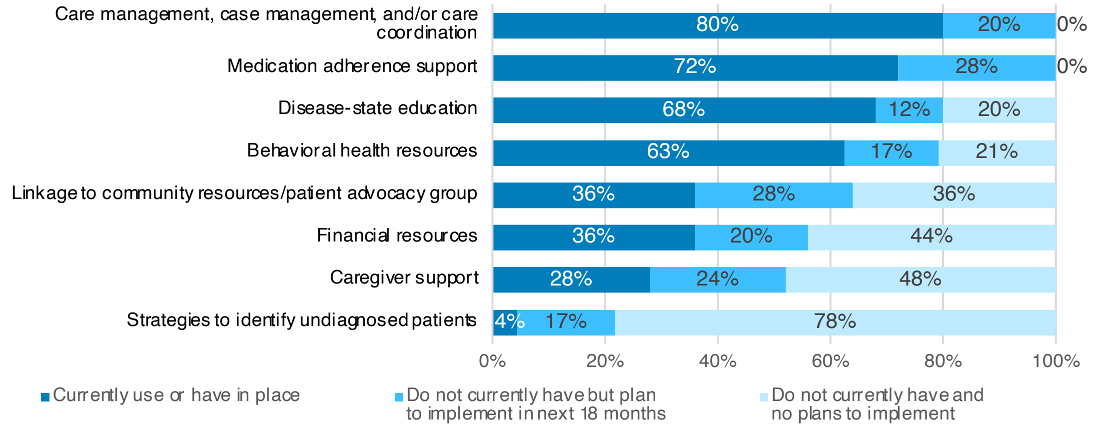


Figure 3: **What resources/programs does your organization provide for patients with rare/orphan diseases? (Provider respondents)**

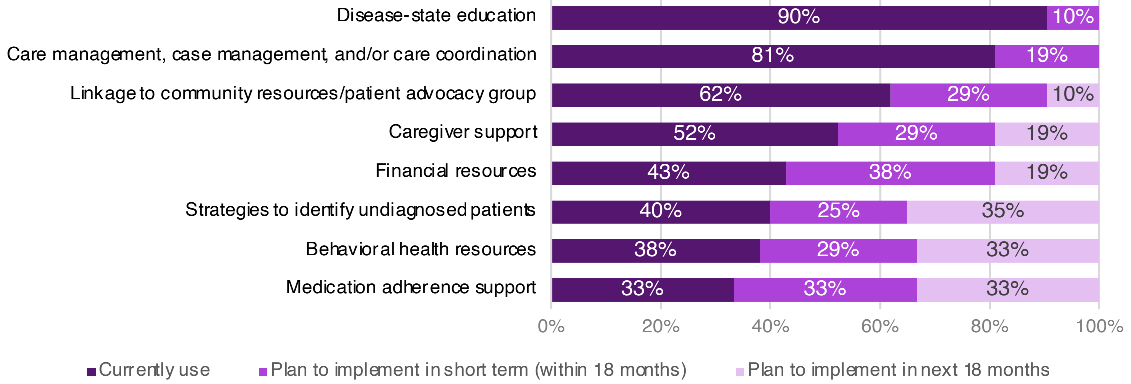
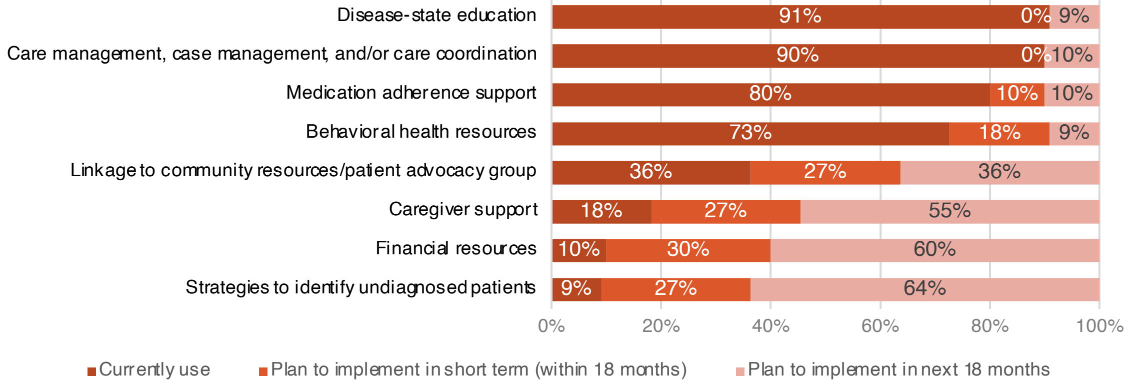


Figure 4: **What resources/programs does your organization provide for patients with rare/orphan diseases? (Employer respondents)**



Conclusions

- While many providers are currently documenting PROs for their rare disease patients, use by payers has been limited
- Care management and disease state education remain the mainstay of resource offerings for rare disease patients across healthcare stakeholders
- There is interest to expand these offerings, particularly caregiver support, linkage to community resources, and financial assistance

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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