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U.S. Commercial Payer and Rare Disease Patient Stakeholder Perceptions of Value of Orphan Drugs

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Background

- Payer stakeholders occasionally utilize existing drug value assessment frameworks, and even less so, direct patient stakeholder input to inform their drug formulary decisions that impact the lives of patients (the customers).
- Patient perspectives on (drug's) value may differ from that of physicians and payers, and even among patients, individual values and preferences may differ. (1,2)
- Patient input on drug's value may assume importance in rare disease arena, where payers encounter incomplete evidence supporting orphan drug value and high prices, while patients suffer disproportionate morbidity/mortality, amidst high disease burden and limited treatment options. (3-5)
- Even if payers have the best of the intentions to help patients in need and make value judgement about drugs of interest to patients, the mere lack of direct involvement of patients to understand their value perceptions & preferences may fail to do justice to payer intentions.
- The disconnect between the payer and patient views of therapeutic value sometimes could lead to payers severely restricting or blocking access to medicines that patients are in dire need for.

Objective

- Assess U.S payer and rare disease (RD) patient perceptions of value of drugs in general, and orphan drugs (ODs).

Methods

- In-depth qualitative telephone interviews with 24 payer and patient stakeholders, encompassing:
  - Payer stakeholders*: Medical and pharmacy directors from Managed Care Organizations (MCOs) and Pharmacy Benefit Managers (PBMs);
  - Patient stakeholders: Rare disease patient advocates, adult patients with rare disease and parents of children with rare disease.
- The payer* and patient stakeholder interviews probed the following topics:
  - Consumer (patient) perception of payer reputation, credibility, loyalty, trust, patient-centricity
  - Consumer (patient) knowledge of formulary committee composition and decision process
  - Definition of 'value'
  - Perception of value of orphan drugs
  - Perception of incorporation of patient input/preferences into payer formulary decision-making process
  - Factors preventing payer use of patient input in formulary decision-making process
  - Optimal approaches to soliciting patient stakeholder input on orphan drug's value
  - Optimal approaches to incorporating patient stakeholder input on orphan drug's value
- A constant comparator analysis was adopted to identify emerging patterns of insights and refine the discussion points to probe pertinent topics of interest in the forthcoming interviews.
- Audio transcripts of the interviews were coded using MAXQDA software for qualitative analysis.

**Focus was on U.S commercial/private payers.*

Limitations

- Small sample sizes may limit generalization of results. This research is geared towards hypothesis generation to fuel further research and discussions concerning payer consideration and incorporation of rare disease patient input in payer formulary decision-making process.

Disclosure

- Authors are employees of Avant Health LLC. There are no conflicts of interest to declare.

References

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Results

Table 1: Patient Stakeholder Characteristics	N=17
Age, mean years	45.9
Female, %	64.7
Years living with a RD, mean years:	
Adult patients with RD	24.2
Children with RD	7.1
Experienced issues accessing RD medications for themselves or for their children with a RD, %	41.1
Living with RD condition (adults, or their children), %:	
Duchenne	35.3
Hypoparathyroidism	23.5
Spinal Muscular Atrophy	17.6
Fabry Disease	17.6
Tay Sachs	5.9

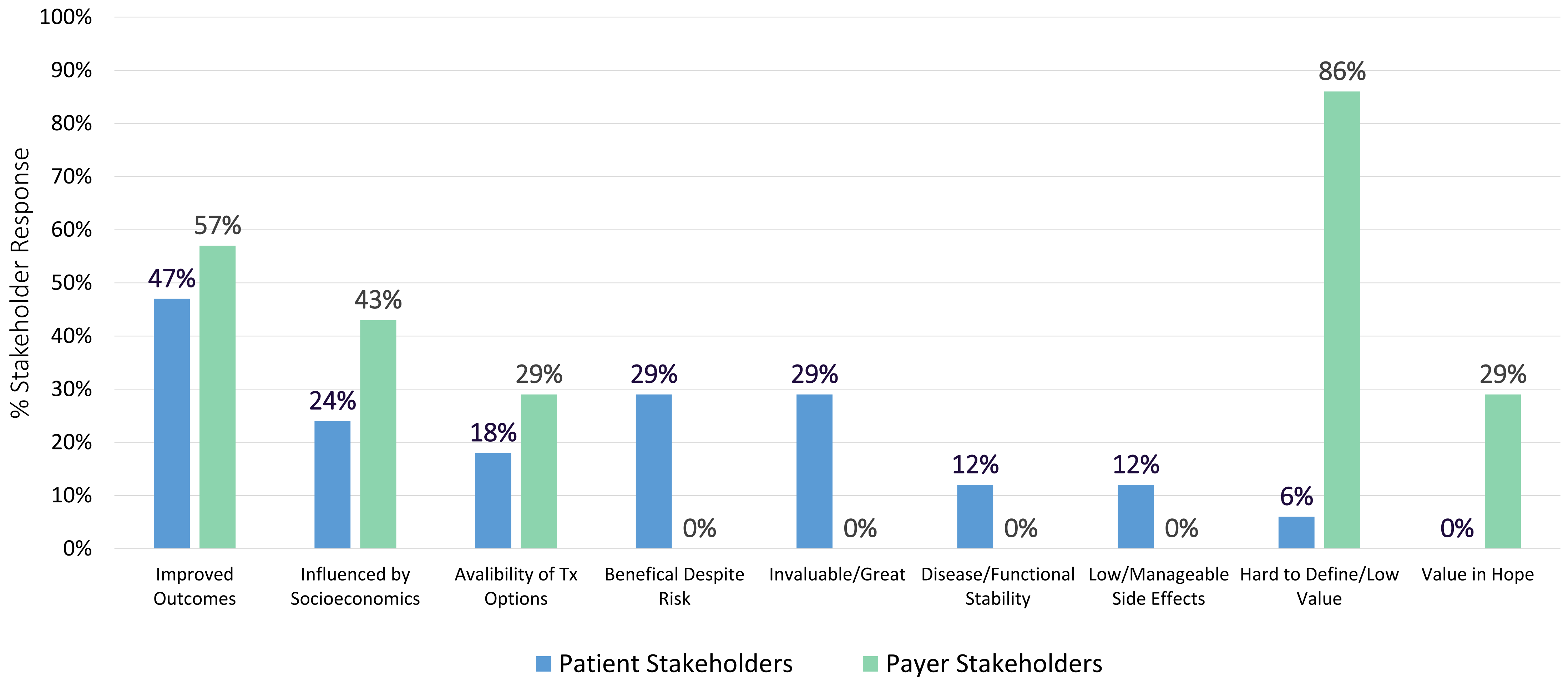
Table 2: Payer Stakeholder Characteristics	N=7
Age, mean years	45.7
Years working in a private health plan or PBM setting, mean years	18
Experience within P&T / formulary committee, mean years	12.9
Size of private health plan or PBM, mean covered lives	1.89 million

- When defining drug's 'value' patients and payers have different, albeit, overlapping perceptions. Patients identified treatment efficacy (47%), impact on QOL/ADLs (41%), and affordability (29%) as key attributes defining drug's value. Payers overwhelmingly (86%) identified "outcome per cost" while defining drug's value; one payer noted:

> *"Value is about whether something is worth it. When I think about value, I usually think about how much we're paying to achieve a particular goal and whether that is realistic or not; the value in a pharmaceutical is what it does for patient outcomes." – C18 (Payer)*

Note: ADL: Activities of Daily Living; QOL: Quality of Life; RD: Rare Disease; P&T: Pharmacy and Therapeutics; PBM: Pharmacy Benefit Manager.

Figure 1: Perception of Value of Orphan Drugs - Patient and Payer Stakeholders



N=24 , Tx: treatment

Table 3: Stakeholder Comments Regarding Value of Orphan Drugs
"This drug has given me a quality of life. I placed great value on it. It's a million-dollar drug. It's making me feel like Wonder Woman. The value I put on it is how it makes me feel. It makes me feel much better than I did when I didn't have the drug." - C3 (Patient)
"You know, I'm willing to have the muscle biopsy if it means getting them [children] on this drug that I know is working."- C16 (Caregiver)
"… not being able to have mobility versus little mobility… I'd rather stay stable." – C5 (Patient)
"Quality of life is over quantity for me. So, I want to make sure that he has a good quality of life." – C13 (Caregiver)
"If it's a novel approach, first in class, then really, it's more of an arbitrary acceptance of its cost." – C19 (Payer)
"With these orphan conditions, it's very challenging [to define value]. If there's something like less than 50 patients in the US. So obviously, to treat something like this, the cost is going to be very high on a number basis." – C22 (Payer)
"One patient can bankrupt our financials for a given year." – C23 (Payer)

- Patient stakeholders and payers ranked different OD attributes as high or very high, as shown in **Table 4**. Majority of patients ranked impact on QOL as high/very high, whereas majority of payers did not mention this attribute at all.

Table 4: Stakeholder Ranking of Orphan Drug Attributes

Ranking	Effectiveness		Safety		Impact on QOL		Treatment Complexity		OOP Cost		Overall Evidence	
Stakeholder	Patient	Payer	Patient	Payer	Patient	Payer	Patient	Payer	Patient	Payer*	Patient	Payer
High/Very High	82%	86%	65%	43%	76%	14%	35%	0%	35%	57%	59%	0%
Medium	6%	14%	18%	29%	12%	14%	12%	29%	18%	0%	6%	57%
Low	0%	0%	0%	0%	0%	0%	35%	43%	35%	29%	0%	14%
No Comments	12%	0%	18%	29%	12%	71%	18%	29%	12%	14%	35%	29%

*Refers to overall treatment cost for payers. QOL: Quality of Life; OOP – Out of Pocket.

Conclusion

- There are considerable differences in patient/payer perceptions of drug's value, and most specifically, value of ODs. Payer recognition of these differences and periodic assessment and incorporation of patient stakeholder perceptions in their formulary decision-making process could improve OD formulary decisions and enhance customer perception of payer's patient-centricity.