



Commercial payer and rare disease patient stakeholder perspectives on incorporation of direct patient input on value of medicines in payer formulary decision-making process in the U.S.

AVANT HEALTH

Democracy Plaza
6701 Democracy Blvd, Ste 300,
Bethesda, MD 20817, USA

S. Narayanan. Avant Health LLC, Bethesda, United States

Siva Narayanan:
W: +1 301.799.8268
E: SNarayanan@Avant-Health.com

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 payer and rare disease patient perception towards payer reputation, patient-centricity, orphan drug value, payer-patient engagement, and incorporation of rare disease patient perspectives on value of orphan drugs in drug formulary decision-making process in the U.S.

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.

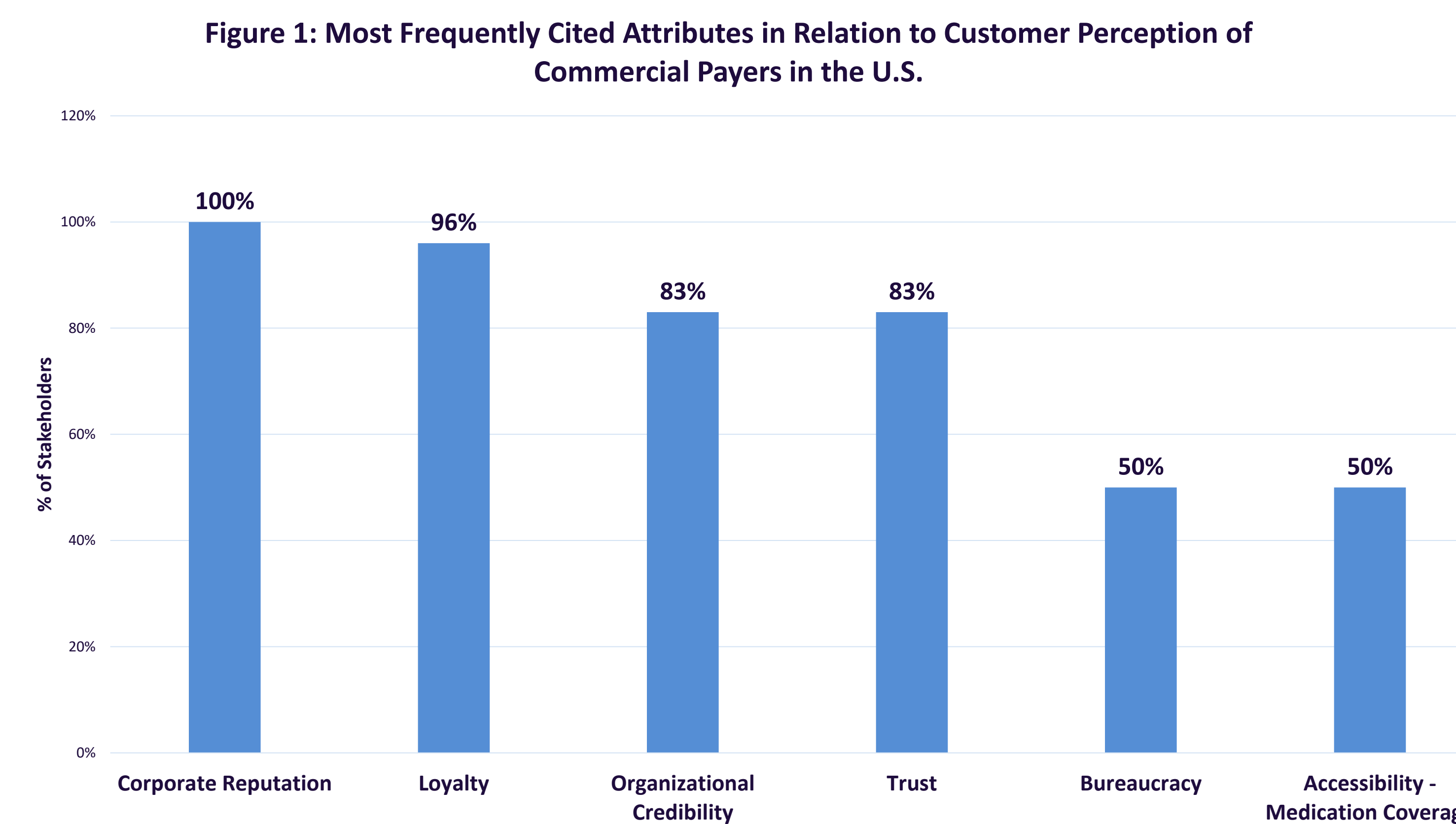
*Focus was on perceptions about commercial payers

Results

- Payer stakeholders had an average of 13 yrs (range: 5-30 yrs) of experience in P&T committees.
- The average size of payer organizations in terms of covered lives represented in the research was 1.89 million (range: 50,000 – 45 million).

Results

- Average age of patient stakeholders was 46yrs (range: 21-63yrs); two-thirds of them were female.
- The adult patient stakeholders had an average of 24.2 yrs. of experience living with a rare disease, and the pediatric patients had an average of 7.1 yrs. since diagnosis of a rare disease.
- Slightly less than half of the patient stakeholders (41.1%) reported experiencing some issues accessing rare disease medications for themselves or for their children with a rare disease.
- The rare diseases observed among patient stakeholders included Duchenne, Fabry Disease, Hypoparathyroidism, Spinal Muscular Atrophy, Tay Sachs.
- The top 5 attributes of commercial payers frequently cited by interviewed stakeholders are shown in figure 1.



- 44% of patient stakeholders and 86% of payer stakeholder identified payers as patient-centric; 41% of patient stakeholders noted payers to be economically driven, instead of being patient-centric.
- 88% and 82% of patient stakeholders noted their lack of knowledge of payer formulary committee composition, and its decision-making process, respectively.
 - Only 24% of patient stakeholders acknowledged that health care professionals such as doctors may be a part of the decision-making committee/process.

Perception of Orphan Drug Value

- Majority (76%) of patient stakeholders identified several orphan drug attributes delivering value; 29% noted orphan drug as invaluable/outstanding.
- Majority (86%) of payer stakeholders noted orphan drug value is low or hard to define; 29% also mentioned cost is secondary when there are fewer treatment options for rare disease(s).
- Both stakeholders valued improved outcome as an important orphan drug attribute, with patient stakeholders mentioning that quality of life is more important than the quantity of treatment options & side effects, and payer stakeholders indicating that value is weighed more towards improved outcomes and efficacy.

Conclusion

- **Formulating a mechanism to formally incorporate patient input in payer formulary decision-making process could benefit payers by enhancing patient (customer) perception of payer's patient centricity and reputation, influencing credibility, trust, and customer retention.**

Consideration of Patient Stakeholder Input in Formulary Decisions

- 88% of patient stakeholders stressed the importance of considering patient input in formulary decisions.
- 86% of payer stakeholders indicated their skepticism/undesirability in considering patient input.
- Both stakeholders noted the benefit of patient input in reflecting orphan drug benefit/risk and its impact on patients, supporting value judgments.
- 53% and 71% of patient and payer stakeholders respectively noted negative perceptions payers hold towards patient's direct input is preventing payer consideration of patient input.
 - Payer attributes: Internal financial pressure, lack of outlet for patient stakeholders to share input.
 - Patient attributes: Patient stakeholder input considered less reliable due to subjective nature

Key Potential Sources of Patient Input as Mentioned by Stakeholders	
Patient Stakeholders	Payer Stakeholders
Surveys	Patient Advocates
Interview/Focus Groups	Disease Advocacy Groups
Written Correspondence	HCPs & Internal Case Mangers

- Incorporation of patient input into formulary decision making process is considered to enhance payer's patient centricity, credibility, trust, and reputation (figure 2).

Figure 2: Incorporation of Patient Stakeholder Input & Associated Benefits



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.

Disclosures

There are no conflicts of interest to declare.

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