Monarch Business School Switzerland

Ph.D. in Business Research
Dissertation Summary

Value of Medicines:
An Investigation of Incorporation of Patient Input into Payer Decisions in the U.S.

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May 2022

Abstract

Private payers in the U.S undertake drug formulary deliberations routinely to evaluate the value of drugs in different disease areas and decide on their inclusion in drug formulary, which would then make the drug available for patients in need. Payers predominantly consider benefit/risk ratio and treatment cost into their value equation, and seldom consider patient input/preferences and their perception of drug value. The lived experiences of patients as a function of the impact of disease on their health and the impact of medicines to manage their disease burden directly influence their perception of true value of medicines; patient's value perceptions tend to differ from those of payers and healthcare providers, especially in rare disease (RD) arena, resulting in gaps in stakeholder expectations, behavior (value judgments) and outcome (drug inclusion in the formulary).

The present executive summary abridges the research findings of the doctoral research submitted to Monarch Business School Switzerland on "Value of Medicines: An Investigation of Incorporation of Patient Input into Payer Decisions in the U.S". The introduction presented the background of the problem. In the Literature review, influential authors in the domains of value assessment frameworks (VAFs), ethical decision making (EDM), and corporate social performance (CSP) theories have been reviewed and critiqued. The methodology and data presentation exhibited the research method, design and data collected during the field work component. The theoretical and applied contribution to the domain were revealed in the synthesis and integration. The conclusion indicated recommendations for implementation and identified areas for future investigation.

The research focused on the nexus within the academic literature with respect to VAFs, EDM and CSP theories to understand the factors influencing the current payer decision-making process and the lack of incorporation of patient input into that process. The final aim of the research was to develop a pragmatic framework for payer-patient engagement that facilitates incorporation of patient input into payer drug coverage decision-making process in the U.S.

The research was believed to be the first to solicit payer and patient perspectives on their drug value perceptions, payer decision-making process, value of consideration of patient input regarding RD drugs, and modalities to incorporate such input into payer decision-making process. In light of utter absence of past research on these topics, this research fills a critical void in the academic literature and offers a pragmatic framework for payers to implement in their organizational settings to strengthen their formulary decisions, improve their social performance and reputation, as well as benefit the customers (patients) they stand to serve.

Keywords: Drug formulary, value assessment, ethical decision making, corporate social performance, patient input, patient preferences.

1. Introduction

Payer drug formulary decisions in the U.S are influenced by: (1) published evidence on drug performance, (2) organizational incentives, controls and economics that may drive the organizational bottom line, and (3) market forces related to current/perceived market share of the concerned drug, associated budget impact and the discount offered by drug manufacturers (Dranove et al., 2003; Suh et el., 2002). Specifically in the RD arena, majority of payers identify clinical data of the drug (i.e., safety, efficacy) as the most important factor influencing their drug formulary coverage restrictions, with drug cost being the second most important factor (Hyde & Dobrovolny, 2010; Quinn, 1999); most notably, consideration of drug's impact on patient quality of life (QOL) and activities of daily living (ADL), and a general consideration of patient input/preferences are lacking. Perspectives of RD patients however portray a complex picture as they consider drug attributes; patients rate the "chance that the drug will work" as the most significant attribute driving their preference for a drug, followed by the ability to carry out ADLs, and serious side effects (Hyde & Dobrovolny, 2010).

The aforementioned literature hint at some alignment in payer and patient stakeholder consideration of RD drug attributes such as efficacy/safety when assessing its value, while discordance remain in the consideration of patient-centric factors such as drug's impact on QOL/ADLs that are best articulated by patients themselves. Even if payers have the best of the intentions to help patients in need and make value judgement about medicines of interest to patients, the mere lack of direct involvement of patients or solicitation of their input to understand their unmet needs and drug value perceptions and preferences, as evidenced by the lack of literature on this topic, may fail to do justice to their (payer) moral intentions, impede payers in fully recognizing the moral issues associated with the impending formulary decision, and thereby potentially influencing their moral behavior (of judging the drug's true value and its inclusion in the formulary). Besides considering all available evidence, factors such as committee member knowledge/experience, organizational sensitivity to drug budget impact thresholds may influence their ethical decision making. The processes of information gathering, stakeholder assessment/engagement and (RD drug coverage) issue management and efforts to align with external stakeholder needs/preferences by narrowing any expectational gaps could improve social performance of payers in the eyes of customers (patients), thereby potentially influencing factors such as credibility, trust, and corporate reputation. On the contrary, when access to RD drugs are restricted by payers without considering patient's value perceptions and how patients may in the future or currently benefitting from the drugs, patient community face dire consequences impacting their morbidity and mortality and they struggle to comprehend the access issues and strive to get their voice heard (Perfetto et al., 2017; Mulberg et al., 2019;

Rodriguez-Monguio et al., 2017; Uhlenbusch et al., 2019). Such patient experience could further feed into negative perceptions (of social performance, and reputation) patients/customers already hold towards payer/health insurance industry in the U.S (DiJulio & Firth, 2015).

Research characterizing stakeholders' perceptions towards patient preference in decision making along the medical product lifecycle highlighted healthcare provider (HCP) skepticism/doubts about patient's ability to articulate product's value owing to their lack of scientific knowledge, low quality preference methods and the lack of fit within current decision-making processes (Janssens et al., 2019). There is however an absence of any research on payer and patient stakeholder perceptions of the benefits of consideration patient input in payer formulary decisions, and the factors that may potentially impede such consideration. Further, an evaluation of patient stakeholder awareness of payer decision-making process, and a direct comparison of payer and patient perspectives on value of medicines in the context of drug formulary decisions has not been adequately studied. Research filling these literature gaps and an accompanying pragmatic framework for direct payer-patient engagement and modalities by which payers can incorporate patient input could all help payers transform themselves into patient-centric organizations and improve their social performance, thereby shedding the negative public perception and improve their corporate reputation.

2. The Main Research Question

With the aforementioned literature in mind, the following main research question has been developed:

The Main Research Question

> *"What are the characteristics of a new conceptual framework that would facilitate the incorporation of patient input concerning 'value of medicine' into the private payer drug coverage decision-making process in the rare disease arena within the U.S. healthcare system?"*

Payer-patient engagement lacks a coherent framework, and a framework for incorporating patient input into payer decision-making process is utterly lacking. The theoretical framework was hence structured based on an integrative literature review and content analysis, as a nexus of three theories, encompassing value assessment frameworks (VAFs), ethical decision making (EDM) theories, and corporate social performance (CSP) theories. A comparison of modalities of patient engagement in

payer decision-making process in other countries such as the U.K, and Canada was included as a practical domain to complement the three theoretical domains to evaluate the research phenomenon of interest.

TABLE 1
Bibliometric Analysis

	Google Scholar	JSTOR
SEARCH TERMS	**SEARCH RESULTS**	
1. Drug value assessment in the U.S.	4,040,000	83,974
2. Ethical decision making in the U.S.	2,620,000	206,531
3. Corporate social performance in the U.S.	3,620,000	171,537
4. Patient engagement in medical decisions in the U.S.	384,000	16,671
5. Payer drug coverage decisions in the U.S.	57,800	1,877
6. Search terms # 5 + # 1	39,700	828
7. Search terms # 5 + # 2	28,600	533
8. Search terms # 5 + # 3	26,600	523
9. Search terms # 5 + # 4 + # 2	22,800	155
10. Search terms # 9 + Private payers in the U.S.	17,600	46
11. Payer-patient engagement to aid drug formulary decisions in the U.S.	110	20

Source: Narayanan (2022)

The evaluation of published literature as it pertains to drug value assessment, EDM and CSP in general yielded adequate results, as shown in Table 1. Evaluation of these topics in the context of private payers in the U.S and their drug coverage decisions yielded a smaller number of search results, and the topic of 'Payer-patient engagement to aid drug formulary decisions in the U.S.' yielded minimal results, further highlighting the scarcity of research on patient engagement in private payer decisions in the U.S., and the potential significance of this contemplated research in contributing to the external body of literature.

Table 2 Definition Of Terms	
Term	**Definition**
Caregiver	An individual who takes care a family member (especially, a son, daughter, spouse. partner) with a debilitating disease condition and needing assistance with daily activities (Perfetto et al., 2017). For the purpose of this thesis research, this will be the parent of a pediatric patient with a rare disease.
Formulary	A formulary is list (of drugs, devices and healthcare services) that is created and updated periodically by payer organizations to reflect their level of coverage and reimbursement in concerned disease conditions and relevant markets. Decisions to include drugs, devices and healthcare services in the formulary is based on the clinical judgment of HCPs and healthcare administrators affiliated with payer organizations (ASHP, 2008).
HCP	HCP refers to physicians, nurse practitioners, pharmacists and other allied healthcare professionals who provide care to patients.
HTA	The Health Technology Assessment, commonly known as HTA, is the systematic evaluation of drugs, devices, and healthcare services to assess their value and impact on patient's health as a result of use in usual care settings. This evaluation is expected to inform adoption and reimbursement of the concerned interventions in relevant markets (Banta, 2009; WHO, 2020).
Formulary Committee / P&T Committee	A committee within payer organizations that makes formulary decisions concerning the inclusion/exclusion of drugs, devices, and healthcare services. The committee usually comprises of HCPs (incl. physicians, pharmacists, nurses) and healthcare administrators who assist with evaluation medication utilization (ASHP, 2008).
Patient	A patient is a consumer, as well as a customer of payer organizations. He/she has a disease/disability and seeks treatments to manage their condition (Perfetto et al., 2017).
Patient Advocacy Group	An organization or group dedicated to advocacy for patients, survivors, and caregivers, often concerned with a specific disease or a group of disorders. The leaders or champions within these organizations are referred to as patient advocates.
Patient Stakeholder	Patient stakeholder refers to individual patients and their family members, and patient advocacy group members, for the purpose of this dissertation research.
Payer	Payer is an organization which manages the financial and operational aspects of health care delivery related to its members (or subscribed customers). Payer category includes health insurance plans or managed care organizations (MCOs), pharmacy benefit managers (PBMs), and certain Accountable Care Organizations (ACOs). For this dissertation

	research, payers shall exclusively refer to private payers, which are private insurance companies (the MCOs) and PBMs in the U.S.
Rare Disease (RD)	In the U.S., RD is any condition that affects < 200,000 individuals. (NIH, 2017).
RD Drug / Orphan Drug	RD drugs are also called Orphan drugs; Orphan drugs are generally defined as "those medicines with one or more indications approved under the Orphan Drug Act, indicated for treating, preventing or diagnosing rare or ultra-rare diseases". (NCI, 2011)
Value	For the purpose of this dissertation research, from patient's perspective, drug's value is defined as 'how well the drug works in alleviating patient burden and in improving QOL, and the balance between its benefits versus risks.'
Value Assessment Framework (VAF)	An approach to assessing the value of a treatment by capturing the benefits, risks (or potential harms), and total costs in a systematic and evidence-based way (ICER, 2020; Perfetto et al., 2017).
Source: Narayanan (2022)	

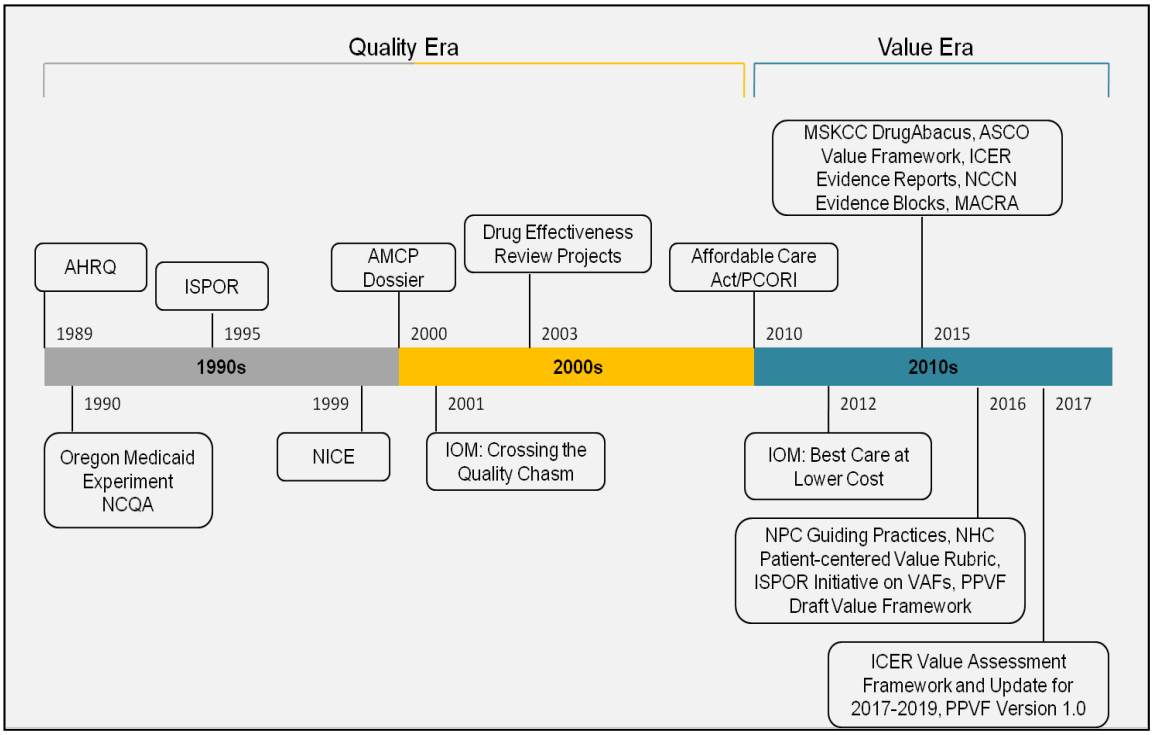
Table 2 aggregates definitions on terminology. The heterogeneity of RDs, variation in personal value system of individuals with RD, and variation in RD drug prices were identified as potential limitations of the research, impacting the generalizability of the research. The narrow focus of research on private payers, and the focus on medical and pharmacy directors serving in the payer formulary committees were identified as delimitations of the research, even though, the defined scope of research sample was expected to offer clarity on interpretation of results.

3. Literature Review

The literature thematically reviewed the seminal authors whose contribution is relevant in addressing the research phenomenon and the associated theoretical domains. The origins, strengths and drawbacks of the six VAFs, namely, ACC-AHA value framework, ASCO value framework, ICER value framework, DrugAbacus, NCCN evidence blocks and Patient-perspective value framework, are reviewed (Seidman, 2017; Snow et al., 2020). Specific focus is applied to these framework elements concerning evaluation of the value of therapeutic interventions (i.e., drugs), how patient preferences and input are incorporated into these processes, and to what extent payers leverage these frameworks to inform their drug formulary decision-making. The origins and facets of EDM theories, and the seminal works of Kohlberg (1969, 1981, 1984), Rest (1974, 1986), Travino (1986), Jones (1991), Reynolds (2006), and Sonenshein (2007), and their impact on the evolution of EDM theories are highlighted; the facets of EDM theories that are relevant to payer organizations and their actions are elucidated. Further, the origins and facets of CSP theories, seminal works of Preston and Post

(1975), Ackerman (1975), Sethi (1975, 1979), Carroll (1979, 1991), Wartick and Cochran (1985), Wood (1991), Wood and Jones (1995), and Husted (2000) and their impact on the evolution of CSP are highlighted; the facets of CSP theories that are relevant to payer organizations and their actions are elucidated. Finally, the modalities of patient engagement in payer decisions in the U.K and Canada are reviewed, with the goal to identify the best practices that could be adopted for the U.S.

FIGURE 1
Evolution of Value-based Healthcare in the U.S.



Note: AHRQ – Agency for Health Care Research and Quality; AMCP – Academy of Managed Care Pharmacy; ASCO – American Society of Clinical Oncology; ICER – Institute for Clinical and Economic Review; IOM – Institute of Medicine; ISPOR – International Society for Pharmacoeconomics and Outcomes Research; MACRA – Medicare Access and CHIP Reauthorization Act of 2015; MSKCC – Memorial Sloan Kettering Cancer Center; NCCN – National Comprehensive Cancer Network; NCQA – National Committee for Quality Assurance; NHC – National Health Council; NPC – National Pharmaceutical Council; PCORI – Patient-Centered Outcomes Research Institute; NICE – National Institute for Health and Care Excellence; PPVF – Patient-Perspective Value Framework.

Source: (Snow et al, 2020, p. 9)

TABLE 3
Comparison of Value Assessment Frameworks in the U.S.

	ASCO	NCCN	Drug Abacus	ACC-AHA	PPVF	ICER
Key Attributes	**Audience: Physicians & Patients**					**Audience: Payer**
Therapeutic Benefit	√	√	√	√	√	√
Patient Benefit / QOL	√				√	√
Drug Price	√	√	√	√		√
Drug Cost, incl. OOP Cost	√				√	√
Drug Cost-effectiveness						√
Drug Budget Impact						√
Patient Perception of Value					√	√

Source: Narayanan (2022)

3.1 Genealogical Observations

The evolution of the value-based healthcare in U.S. is depicted in Figure 1. Value assessment has been a critical pillar of healthcare delivery in the U.S. In the two decades prior to 2010, commonly referred to as the 'quality era', the U.S. healthcare system saw the elevation of AHRQ and NCQA into the mainstream of care delivery evaluations, along the with the proliferation of ISPOR and AMCP organizations that advocated for efficiency in care delivery. The introduction of Affordable Care Act and PCORI with a mandate to ensure quality and assess value in care delivered, the 'value era' commenced in the U.S. and reached its peak in 2015-2016 when several therapeutic VAFs were introduced. One common theme across the VAFs is that the therapeutic benefit and drug price or cost are considered by all the VAFs; besides, there is inadequate consideration given to patient-centric attributes that are important to patients and their caregivers and their preferences (tied to the perception of drug value), as depicted in Table 3. An analysis comprising of 101 payers in the U.S. found that only 53% expected that the existing VAFs will have an effect on their internal drug value assessment procedures in the near future (Capuano et al., 2016; Green et al., 2016; Seidman, 2016; Slomiany et al., 2017). In another research with 11 U.S. payers, between 64-82% of them reported unlikely to use VAFs, whereas less than one in ten payers reported that they will very likely use the VAFs a year from now, to aid their formulary decisions (Seidman, 2016). With this lackadaisical consideration of VAFs by payers in their drug coverage decisions around the country, coupled with the inherent absence of consideration of patient value perceptions within VAFs in first place, in-depth conversations with payer stakeholders are warranted to improve the understanding of payer decision-making process, their mechanisms of evidence deliberations, and

current impediments to involving patient stakeholder input in their decision-making process. Such stakeholder input could be leveraged to develop a framework for payer-patient engagement and incorporation of patient input into payer decisions in a pragmatic and sustainable manner.

The second major focus of the literature review was EDM theories, to contextualize and unearth the complexities associated with decisions made by stakeholders (mostly, HCPs) within payer formulary committees as they are influenced by the nature of evidence, ability to interpret it, and the ability to apply organizational and personal value system and experience to guide the decisions. EDM models based on cognitive developmental approach to making ethical decisions posits that individuals' responses to ethical issues are "always based on deliberate and extensive moral reasoning" (Sonenshein, 2007, p. 1022); they provide an organizing framework involving the consideration of affect, behavior, the self-concept, and social information as they together influence an individual's morality (Kohlberg, 1969, 1981, 1984; Rest, 1986; Rest et al., 1974; Trevino, 1986). Building off of these, Jones' issue-contingent model postulated that the ethical issue is intuitively judged based on the degree of moral intensity, influenced by the specific situation/context; organizational factors however may influence establishing moral intent and engaging in moral behavior, thereby impacting decisions (Jones, 1991). The theoretical overlap of this EDM model with VAF domain posits that as organizations recognize moral issues (e.g., disease burden and unmet treatment needs of RD patients), undertake moral reasoning (to include an orphan drug in the formulary, in the context of limited treatment options), and express intent to make the right decision, the organizational behavior of EDM may be influenced by organizational factors such as corporate policies/procedures in managing high price drugs in formulary, pre-set budget impact thresholds, and the group dynamics (in evidence consideration and deliberation) within the formulary committee. There is no literature depicting these relationships in the context of U.S payers.

The neurocognitive model of EDM theorizes the comparison of new situation to pre-existing, experience-based knowledge (or prototypes), enabling an automatic recognition of ethical dilemma and how society may interpret it; in the absence of protypes, active judgement is engaged (Reynolds, 2006). The theoretical overlap of this model with VAF domain posits that decision makers (e.g., formulary committee members within payer organizations) rely on their pre-existing experience-based knowledge (in RD value assessment, disease burden recognition, and evidence synthesis) to judge the issue in hand (e.g., drug's value) and aid decision (surrounding inclusion of a drug in their formulary). Considering the diverse and unique nature of a RD, comprehension of the issues specific to an RD to inform decisions may prove challenging to committee members. There is currently scarce literature on payer

deliberative process in RD drug value assessment, and the tools/information they leverage to support active judgement that may fuel their drug formulary decision.

Finally, the sensemaking model of EDM posits that if a situational appraisal is perceived to have an ethical implication, a process of self-reflection to forecast outcomes associated with different courses of action may take place, impacting decision-making (Mumford et al., 2008). The theoretical overlap of this model with VAF domain posits that as the decision-makers (e.g., payers) conduct evidence synthesis in the concerned RD arena with high unmet need and realize the ethical implication of drug formulary decision, they may forecast the implications of formulary inclusion/exclusion of the orphan drug (e.g., improved population health outcomes, organizational budget impact), before arriving at the final coverage decision. There is limited literature depicting the dynamics of payer decision-making in RD arena, incl. the trade-offs considered by payers during their drug value assessment

The third major focus of the literature review was CSP theories. CSP is defined as principles of an organization to convert their actions and achieve respect and image through their outcomes of those actions (Wood, 1991). CSP concepts have evolved overtime from the emphasis on organizational actions governed by ‘self-interest’ to gain superior financial performance, to instead focus on principles, processes and outcomes, with outcomes attribute encompassing "internal stakeholder effects, external stakeholder effects, as well as external institutional effects,” as outlined by Stakeholder perspectives of CSP (Wood, 1991; Wood & Jones, 1995). The theoretical overlap of this framework with VAF domain posits that organizations (e.g., payers) may adopt policies (e.g., RD drug value assessment and coverage policies) considering its social impact and stakeholder interests/needs. External stakeholders (customers/patients) evaluate organizations based on how well organizations have met their expectations and/or how the organization’s behaviors have affected them or other stakeholders and organizations in their environment, and consequently judge firm’s outcomes. These issues may shape customer perception of (payer) organization’s social performance and formulate their perception of loyalty, trust, and credibility, thereby improving reputation and customer retention. These topics have not been explored in the context of payer organizations in the U.S and their customers (patients). Issue contingent model of CSP on the other hand posits that a firm’s response to social issues (corporate social responsiveness) should depend on the nature of the social issue (e.g., unmet needs) and the expectational gaps between the firm and its customers/society related to firm’s CSP (Husted, 2000). The theoretical overlap of this CSP model with VAF domain posits that if a (payer) organization searches for new information about alternative solutions by gathering all relevant information (on drug’s value) at its disposal to reach agreed-upon goals (e.g., optimal drug coverage), and/or if it engages its stakeholders and jointly

resolve contentious issues (e.g., perception of drug's value and its inclusion in formulary) through a process of negotiation and align on their expectations, and/or if an organization questions its prevailing policies to develop a new vision of the organization-stakeholder relationship (via direct customer engagement) aligned with moral reasoning, (payer) organization's CSP is expected to be high, resulting in their improved reputation, legitimacy, credibility, and customer (patient) trust/loyalty, and commitment/retention. There is no literature depicting these relationships in the context of U.S payers. High CSP and improved loyalty, trust, credibility and reputation are bound to improve an organization's overall financial performance (Laufer, 2003; Lusambo, 2017; Orlitzky et al., 2003; Orlitzky, 2005; Siniora, 2017; Smith, 2009; Tehemar, 2012; Turban & Greening, 1997; Waddock & Graves, 1997; Walker, 2010).

An appropriate balance in consideration of all CSR principles (at both institutional and managerial level) and stakeholder perspectives and expectations is critical for deriving the full benefits of CSP. Tailoring the process of social responsiveness (namely, environmental assessment, patient stakeholder management, and the issue of RD medication access) with patient stakeholder's expectations and needs related to access to medications may help payer organizations to efficiently allocate/use its resources and achieve socially desirable outcomes (such as reduction in health disparity among its customers), could subsequently result in reduced healthcare claims and elevate the status of payer organization in the eyes of its customer stakeholders; in addition, consideration of patient stakeholder input (on drug value) to align with their expectations and needs, while balancing it with scarce resources at hand and be explicit about how stakeholder input was used during the drug formulary decision-making process could all positively influence payer organizational reputation, stakeholder trust, loyalty and commitment/retention, and eventually, its overall financial performance (Laufer, 2003; Lusambo, 2017; Orlitzky, 2005; Orlitzky et al., 2003; Siniora, 2017; Smith, 2009; Tehemar, 2012; Turban & Greening, 1997; Waddock & Graves, 1997; Walker, 2010). There is scarcity in published literature highlighting these in the context of U.S payer organizations. New research leveraging direct engagement (via qualitative interviews) with payer stakeholders and their customers (patient stakeholders) could serve well to further the research in this field.

As the final part of the literature review revealed, countries such as the U.K and Canada are engaging patients actively in their drug evidence assessments and coverage and reimbursement decisions (CADTH, 2020; Hashem et al., 2018; Mulla, 2017; Staley & Doherty, 2016; Stein, 2016). Predominantly qualitative input from patient stakeholders is used in these countries to solicit stakeholder input, via workshops, and oral and written depositions. These offer viable ideas for payers in the U.S to adopt and strengthen their drug formulary decision process.

3.2 Methodological Observations

Literature on VAF, EDM theories and CSP theories depicted a diverse use of methodologies to unearth the underlying respective phenomenon, based on the research objectives. Quantitative research methods such as surveys employing discrete choice experiments and conjoint analysis have been used to unearth the stakeholder attitude, perceptions, and preferences (Mühlbacher & Juhnke, 2013); such methods however are limited by the pre-constructed standardized instruments or pre-determined response categories into which participant's varying perspectives and experiences are expected to fit, thereby rendering emergence of spontaneous and non-pre-conceived topics impossible (Yilmaz, 2013). Researchers have countered these drawbacks by employing qualitative interviews (unstructured or semi-structured) as an integral part of gathering stakeholder perspectives, as they aid in in-depth understanding of experiences and perceptions by soliciting that information in their own words (Gibbons et al., 2018; LaVela & Gallan, 2014; Yilmaz, 2013). Some key concepts and the associated methodologies used in the literature are summarized as follows:

- Concept: Assessment of payer perceptions and decision-making
    - Methods: Qualitative, semi-structured interviews (Seidman, 2016; Wang et al., 2012).
- Concept: Assessment of patient preferences, in general.
    - Methods: Quantitative structured surveys, qualitative semi-structured interviews and mixed methods, based on research objectives (Gibbons et al., 2018; Gooberman-Hill, 2012; Janssens et al., 2019; LaVela & Gallan, 2014; Mühlbacher & Juhnke, 2013; Shafrin et al., 2017; Zafar et al., 2009)
- Concept: Assessment of patient burden, and perceptions and preferences in the context of value assessment and decision-making.
    - Methods: Qualitative, semi-structured interviews and face-to-face discussions (Chauhan & Sagar, 2021; Gooberman-Hill, 2012; Perfetto et al., 2017).
- Concept: Inductive reasoning, and assessment of 'why' behind stakeholder decisions.
    - Methods: Qualitative methods (Cohen, 1996; Rolfe et al., 2018).

Correspondingly, the authors identified qualitative research method to examine stakeholder (i.e., payer and patient) perceptions and preferences and to assess the 'why' behind their preferences and actions, all of which are directly relevant to the research phenomenon addressed by this dissertation research.

3.3 Research Gaps

A detailed analyses of the three literature domains and the intersection of the domains that were considered most relevant to the research phenomenon of interest, namely, payer consideration of patient input in their formulary decision-making process, resulted in the identification of several research gaps. Evaluation of factors patients and payers consider in assessing treatment options and drug's value, especially in the context of RD conditions and RD drugs remain a key research gap. This could be achieved by adopting a qualitative semi-structured interviews of RD patient and payer stakeholders, probing them on specific topics such as: stakeholder definition of 'value', perception of value of RD drugs, factors influencing patient choice of RD treatment options and factors tied to payer's ethical formulary decisions in the context of available patient unmet needs, available treatment options and value of drug under consideration.

Understanding of payer formulary committee characteristics and their decision-making process, policies and procedures is a research gap; to address this, the following topics can be probed via qualitative stakeholder interviews: stakeholder perception of payer formulary decision process, committee composition and their knowledge/sophistication in literature evaluation that is critical for drug value assessment. To fill the research gap in understanding the payer and patient stakeholder perceptions of each other's needs/expectations and consideration of patient input in formulary decisions, qualitative stakeholder probing of the following topics will prove helpful: general perception of payers and their focus on external stakeholders and issues impacting them (thus, concepts of payer-centricity), current status of payer consideration of patient input to inform payer formulary decisions, the specific scenarios that would warrant such consideration, and factors preventing payer consideration of patient input. Finally, qualitative probing of stakeholder perception of payers in general, ways by which patient input can be solicited and used in formulary decision-making process will fill the critical research gap that is core to the phenomena of interest for this dissertation research. Aligned with these approaches to fill the research gaps, a semi-structured discussion guide was created, and qualitative interviews were conducted; the detailed research methods associated with these steps are outlined in the following section.

4. Methodology

The dissertation's methodology was primarily a qualitative research to unearth the factors currently influencing the payer decision-making in the U.S., the impediments to incorporation of patient input into payer decisions, the role patients and patient advocates could play in payer engagements, and the optimal framework to engage patient in payer decision-making process that would benefit both payers and their

customers (the patients) they stand to serve. The qualitative research utilized semi-structured interviews and follow-up interviews to address the research phenomenon of interest.

4.1 Appropriateness of Method

The method selected was appropriate because of the emergent nature of the phenomenon for which grounded theory has been recommended (Moustakas, 1994; Charmaz, 2006). Individuals with RDs usually experience disproportionate disease burden often owing to limited understanding of disease epidemiology and limited treatment options; it is the lived experience of patients and their caregivers that can shed light into the risks/benefits and thus, the overall value of orphan drugs, in the context of disease condition. Capturing the description of lived experiences (in terms of what and how it was experienced), subtle meanings and personally held beliefs without imposing external thought complexes on the participants can be done using the phenomenological methods that have roots in the Grounded Theory. Correspondingly, and aligned with the methodologies used in past research, qualitative interviews were considered an appropriate method to solicit these perspectives from patient stakeholders.

Understanding the factors that influence payer's formulary decision-making, the process of evidence deliberations, the drivers and barriers to assessing drug value and incorporation of patient input in their formulary committee deliberations may all require an in-depth probing and analysis, considering the facets of grounded theory. Whether the interplay of facets of social responsiveness and corporate reputation and drug formulary coverage decisions (be it controversial or not) is in the minds of payers deserve exploration. Further, aim of the thesis research is to construct an explanatory framework that uncovers a process inherent to the substantive area of inquiry (i.e., patient input into payer formulary decisions). Qualitative semi-structured interviews of payer stakeholders were considered to accomplish these.

4.2 Research Design

The research design was a prospective qualitative research involving patient and payer stakeholders in the U.S., that followed Monarch Standardized Process Flow which included the preliminary literature review, an in-depth literature review of select academic domains (namely, VAF, EDM and CSP theories), the content analysis, the questionnaire design and testing, the semi-structured interview process, follow-up interviews, triangulation of the data and gap analysis and the development of the new framework for payer-patient engagement and payer incorporation of patient input.

Target population included 17 patient stakeholders for the micro group (adult patients with RD, parents/caregivers of pediatric patients with a RD, and a patient advocate) and 7 payer stakeholders for the meso group (medical and pharmacy directors with formulary committee experience within payer organizations). The sample was targeted purposely (per purposive sampling method), aligned with Grounded Theory principles. Using two separate 15-item questionnaires (excluding demographic items) for micro and meso groups, data was collected through in-depth telephone interviews which were recorded and transcribed. Follow-up interviews entailed 15 and 7 participants belonging to the micro and meso groups respectively to focus on stakeholder ranking of importance of medication attributes in the context of its value, to add depth to the evaluation of research phenomenon. Additional participant follow-ups were undertaken to simply confirm the participant's intended meaning, or the interpretation of data/input is reflective of their lived experiences and perceptions.

Validity and reliability of research was ensured following Lincoln and Guba's (1985) trustworthiness categories namely credibility, transferability, dependability and confirmability used for verifying the validity of qualitative methods. Specific strategies were undertaken to ensure that there is confidence in the truth of the findings, the research findings apply to other contexts, findings are consistent and can be replicated, and the extent to which findings are shaped by the respondents and not by bias stemming from the researchers own interests (Krefting, 1991; Lincoln & Guba, 1985). These strategies included:

1. Member checking, to improve credibility of research (Charmaz, 2006; Rolfe et al., 2018);
2. Dense description, to improve transferability and dependability of research (Krefting, 1991);
3. Constant comparative analysis, to improve transferability, dependability (Birks & Mills, 2015; Chun Tie et al., 2019; Corbin & Strauss, 2014; Keith et al., 2017);
4. Reflexivity, to improve credibility, confirmability of research (Krefting, 1991; Lincoln & Guba, 1985; Rolfe et al., 2018);
5. Triangulation of data sources and data methods, to improve credibility, dependability, as well as confirmability (Heale & Forbes, 2013; Knafl & Breitmayer, 1991).

Upon completion of the data collection, the data was transcribed, and in-depth content analysis was undertaken, encompassing concept mapping and coding, utilizing MaxQDA® 2020 Analytics Pro software. All patient and payer stakeholders were individually coded, aligned with the domains the respective questionnaires set out to explore and form a thorough understanding of the stakeholder perceptions of drug

value, payer formulary decision-making process, patient participation in payer formulary decisions, and channeling patient input into payer decisions.

5. Data Presentation

The responses of the 24 participants were divided into two parts Part A – Participant Profiles and Part B – Semi-structured Interviews. Each participant received a code name pertaining to their belonging to a particular stakeholder group and listed numerically as C01 to C17 for the micro group and C18 to C24 for the meso group. The categorization included the following demographics for the micro participants: age, gender, name of RD condition, and # of years living with RD condition, type of health insurance, employment status, past issues with drug access. The categorization included the following demographics for the meso participants: age, gender, area of specialization and years of experience in health insurance industry, title, months/years of experience in P&T committee and number of lives covered/insured by their organization.

The average age of the patient stakeholder participants was 45.9 yrs., and two-thirds were female. The adult patient stakeholders had an average of 24.2 yrs. of experience living with a RD, and the pediatric patients had an average of 7.1 yrs. since diagnosis of a RD; 41.1% of patient stakeholders reported experiencing some issues accessing RD medications for themselves or for their children with a RD. The distribution of RD experience among the patient stakeholders was: Duchenne: 35.2%; Hypoparathyroidism: 23.5%; SMA: 17.6%; Fabry Disease: 17.6%; Tay Sachs: 5.9%.

The payer stakeholders had an average age of 45.7 yrs. and an average of 18.0 yrs. of experience working in a private health plan or PBM setting, and an average of 12.9 yrs. of experience in P&T committee. Owing to Covid pandemic, recruitment of payer sample was constrained, due to payer stakeholder preoccupation with Covid-related issues/priorities (in managing population health). Despite this, a diverse payer sample was achieved, with the average size of the payer organization in terms of covered/insured lives being 1.89 million (range: 50,000 – 45 million); the total covered/insured lives across the 7 payer organizations was 129.44 million, in comparison to the total U.S population of 332.9 million in 2021, constituting a robust 39% population coverage; these characteristics reinforced the diversity, appropriateness, and adequacy of payer sample for this dissertation research, despite the smaller sample size.

Pertaining to the data distillation, from the totality of data collected from both the stakeholder questionnaires, a total of 16 categories were produced based on the

resultant findings, aligned with the purpose of the research and answering the main research question. The data was distributed into tables and tallies to illustrate the research methods, identify and highlight the significance of findings. The tally charts further helped to highlight themes/domains concerning the many factors that influence concepts, perceptions and the dynamics of payer formulary decision process and payer consideration of patient input.

5.1 Data Distillation and Triangulation with Literature

The participant perspectives were analyzed, coded and responses tallied using MaxQDA 2020 Analytics Pro software. The key findings were summarized. The triangulation with the literature on pre-identified theoretical domains were performed, as outlined below.

Patient stakeholder perceptions of payers appeared to be negative in general, and noted suboptimal reputation, trust, credibility, and loyalty. These point to the facets of CSP theories that have roots in stakeholder perspectives, and issue-contingency. According to Wood and Jones' CSP theory with a focus on stakeholder perspectives, (patient) stakeholders act as the source of expectations, defining the norms for corporate behaviour (for payers), and act as recipients of payer actions via the process of social responsiveness (Wood, 1991; Wood & Jones, 1995). Consequently, patient stakeholders judge (payer) corporate actions and outcomes, forming opinions of reputation, credibility, loyalty and trust; these attributes may influence customer retention. Further, if payers adopt a comprehensive strategy for evidence gathering and search for all existing and new information concerning drug's value to reach agreed-upon goals (e.g., optimal drug coverage), engage its stakeholders and jointly resolve contentious issues (e.g., difference in perception of drug's value and justification for its inclusion in formulary) through a process of negotiation and align on patient expectations, and, internally, questions its prevailing policies to develop a new vision of the organization-stakeholder relationship (via direct patient engagement) aligned with moral reasoning, (payer) organization's CSP is expected to be high, improving their reputation, patient loyalty, and retention. These attributes may in turn influence corporate financial performance of payers.

Research revealed lack of transparency of payer formulary decision-making process in the eyes of patient stakeholders. Payers however perceive their formulary decision-making process to be robust and their formulary committee members to be knowledgeable and adequately capable of evidence synthesis to aid decision making. The neurocognitive model of EDM posited that when decision-makers encounter an ethical dilemma (e.g., considering a high priced and yet safe/effective RD drug in

formulary), the individual(s) will compare the new situation to pre-existing, experience-based knowledge. Even if past RD-related prototypes exist, every RD condition is unique and disease/patient burden and treatment options vary for each RD, making the transfer of situational knowledge across RD conditions difficult. In such situations, the neurocognitive model of EDM hypothesizes that decision-makers will then engage in active judgment, an active and deliberate processing of information, which in turn would help individuals rationalize and justify their preliminary intuitions or consciously analyze the situation. To strengthen this approach, payers could undertake staff training, process standardization and inclusion of relevant HCPs with various backgrounds and RD knowledge. Payers could then complement it with specific external steps such as concerted outreach and communication to patients/consumers about their process, composition and sophistication of their formulary committees. Both these steps are critical to reinforce their social performance in the eyes of their (patient) stakeholders and bolster their credibility, robustness and transparency of their formulary decision process that are often questioned by patient stakeholders, and in the process, improve corporate reputation, aligned with the facets of the overlap of VAF and Stakeholder perspectives of CSP.

There is a scarcity of literature comparing patient and payer perceptions of drug's value. Definition and assessment of value of RD drugs in this dissertation research clearly depicted the divide between patients and payers, with patients holding a favorable view of RD drugs, and payers holding a negative view. A significant observation that fills an additional gap in research/literature is the identification of overlap of patient and payer stakeholder perceptions of RD drug attributes/categories, and the alignment of the categories with core concepts of various VAFs (Snow et al., 2020; The Lewin Group, 2016). Another key finding is the difference in stakeholder emphasis on select drug attributes, based on their priorities; for example, patients tend to focus on drug impact on QOL and payers tend to focus on drug's cost and budget impact. Creating an environment to exchange these points of view on drug attributes tied to RD of interest could help payers align on stakeholder views and build a productive dialog and permeate the view of patient-centricity; listening to patient input/preferences could further this goal, aligned with the facets of CSP theories (both stakeholder- and issue-contingent theories).

Patient stakeholders however noted their utter lack of awareness of the factors that payers consider in assessing drug's value in their formulary decisions. Both the stakeholder groups had separately identified treatment efficacy/safety, costs (OOP or treatment costs), impact on QOL/ADLs, and treatment complexity/convenience as some of the key drug attributes while considering the value of drugs. Payer consideration of the broad set of drug attributes in their value assessment during formulary decision-

making process underscores the payer organization's moral intent to perform a behavior (i.e., optimal choice of RD drugs in the formulary) and serves as the best indicator of its motivational readiness to act and engage in a moral behavior (i.e., making of right drug coverage decisions, benefiting patients as well as their bottom line). These approaches are aligned with select facets of issue-contingency model of EDM and their overlap with VAF domain. Payers can leverage the alignment with patient stakeholders by highlighting them in their formulary committee's drug evaluation policies and find a common ground to discuss the differences in stakeholder point of views on other topics, and improve their CSP, aligned with the facets of issue-contingent models of CSP.

Research confirmed payer's lack of consideration of patient input/preferences. This finding may influence patient stakeholders to question payer's moral intent to make the right decision, and their moral reasoning to include relevant RD drugs in formulary, aligned with the facets of issue-contingent models of EDM and their overlap with VAF domain. Taking relevant steps to consider patient input could not only improve payer's EDM, but also improve CSP in the eyes of external (patient) stakeholders and garner associated benefits, as outlined by the Issue-contingency models of EDM and CSP and their overlap with VAF domain.

Payer consideration of patient input elicited the most interest from the interviewees. There was some alignment between the patient and payer stakeholders about the utility and rationale/scenarios for soliciting patient input, that included the depiction of lived patient experiences and drug impact on patients. This is in alignment with the rationale depicted by payer/HTA entities in the U.K and Canada for considering patient stakeholder input in coverage decisions, as discussed (CADTH, 2021; NICE, 2013; Staley & Doherty, 2016). There was an inherent hesitation among payers in considering the subjective input from patients, which is similar to the view expressed by NICE stakeholders in the U.K (Hashem et al., 2018); This hesitation could impede payers from fully recognizing the moral issue in-hand (i.e., unmet needs among RD patients, that are best articulated by RD patients themselves), which may have downstream negative impact on their moral judgment (of patient burden that could be alleviated by relevant RD drug) and moral intent (to include a particular RD drug in formulary), potentially culminating in the potential payer decision (the moral behavior) of non-inclusion of RD drug in the formulary (Jones, 1991). Payers in the U.S could overcome their hesitation (of considering patient input) by using the existing/available (objective) literature evidence as a foundation to add RD patient input/experiences and fill the evidence gaps or enhance the value assessment of RD drugs to inform formulary coverage decisions, aligned with the discovery strategy to achieve high CSP, per issue-contingent model of CSP. Highlighting the very nature of consideration of patient input explicitly in the formulary committee policies and procedures, improvision of value

assessment criteria as a result, and externalizing such information will position payers positively in the eyes of patient stakeholders and improve credibility, trust and corporate reputation, per the overlapping facets of VAF and issue-contingency models of EDM and CSP.

Both payer and patient stakeholders highlighted some pragmatic approaches to solicit patient input on drug's value, while clearly indicating that a formal process/channel for payer-patient engagement currently does not exist. Patient stakeholders advocated for payer consideration of qualitative input from patients directly (via ad boards and focus groups), making it mandatory for payers to consider patient input, and including a patient stakeholder in payer formulary committee deliberations; whereas, payers noted relying on external entities such as the FDA or drug manufacturers to already incorporate patient input in drug development/approval process, and is sceptical of inclusion of a patient stakeholder/advocate in formulary committee in any form. The difference in stakeholder views highlight the need for payers and patient stakeholders to be aware of each other's view and expectations, and for payers to implement socially responsive approaches to bridge the expectational gaps on the issue of consideration of patient input in drug formulary decision-making process, with the ultimate outcome of improving payer's EDM process and achieve high CSP.

6. Synthesis and Integration

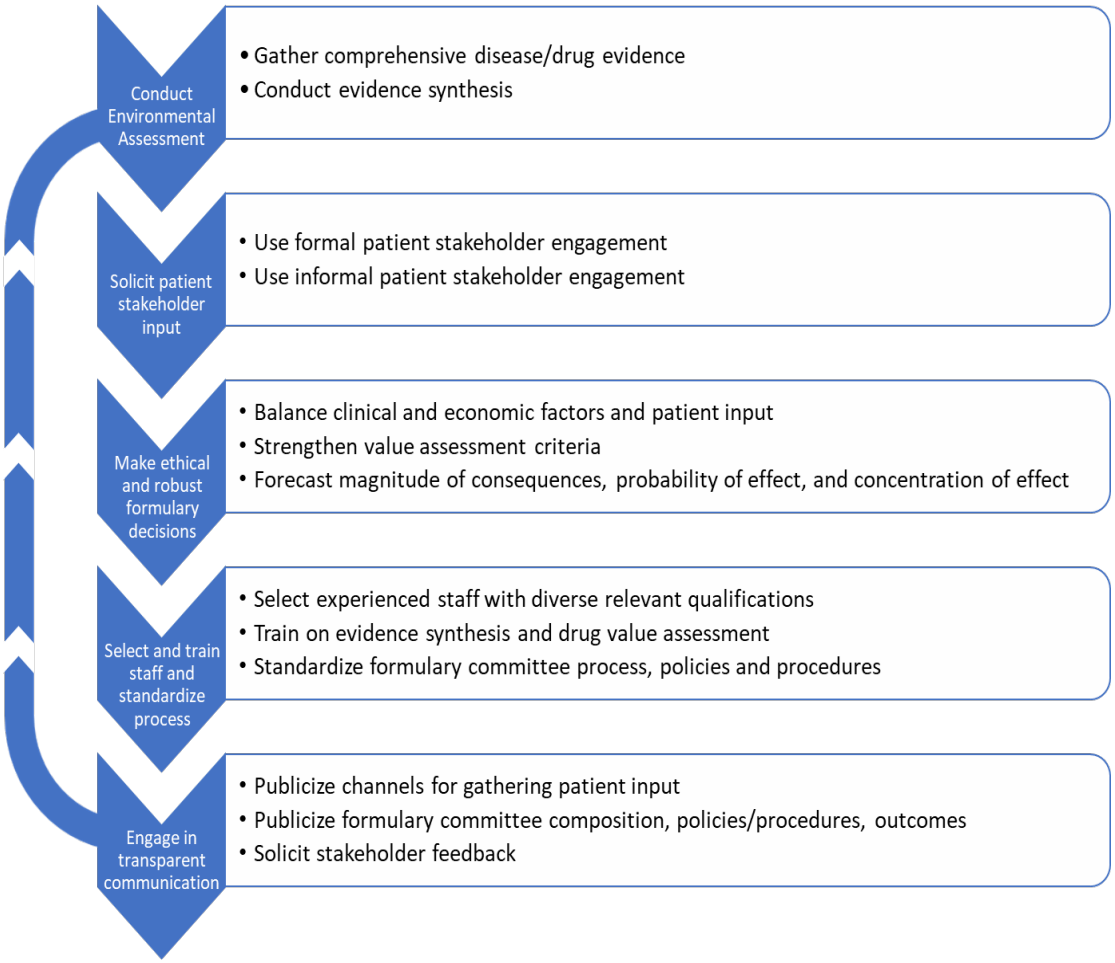
6.1 RD drug value assessment and stakeholder communication

Synthesizing the aforementioned observations and inferences, the following five action themes emerged for payers concerning RD drug value assessment and stakeholder communication, as depicted in Figure 2:

<u>Conduct Environmental Assessment</u>

- Gather comprehensive set of evidence on RD and available treatment options for that RD condition, and mandatorily consider all relevant sources of information for patient input/preferences to understand patient burden and unmet needs and contextualize the moral issue associated with the impending drug formulary coverage decision.
- Conduct evidence synthesis and create disease/drug monographs that are readily available and easily accessible to formulary committee members for review, to aid their deliberations.

FIGURE 2
RD Drug Value Assessment and Stakeholder Communication



Source: Narayanan (2022)

Solicit Patient Stakeholder Input

- Formal patient stakeholder engagement modalities could be introduced by routinely convening patient stakeholder ad boards and focus-groups on relevant RD topics to solicit stakeholder preferences and input surrounding RD drug's value and their lived experiences that could inform drug formulary decisions, especially in case of costly RD drugs. Informal patient stakeholder engagement via solicitation of written or verbal testimonials and anecdotes routinely for all RD drugs could be standardized and incorporated into formulary committee standard operating procedures (SOPs). Payer's RD case managers or customer service

teams could facilitate formal or informal engagement. Engagement of patient advocacy group representatives relevant to the disease condition(s) of interest is critical, and a viable alternative to engaging a smaller sample of individual patients. Leveraging FDA/drug manufacturer materials, treatment guidelines and other literature that incorporate patient stakeholder input could completement direct qualitative input.

Make Ethical and Robust Formulary Decisions

- Robustness of payer RD drug formulary decisions is strengthened by focusing on expanding the breath and comprehensiveness of the drug evidence synthesis beyond published literature and include qualitative patient input concerning their preferences and lived experiences to augment drug's clinical (efficacy, safety, QOL impact, convenience) and economic (cost-effectiveness, budget-impact, OOP) value propositions and understand its true value, from patient perspective. During drug value deliberation process, forecasting the probability of positive or adverse effect of their formulary decisions, the magnitude of consequence of their actions, and the concentration of effect (in terms of size of the RD population impacted, or the organization's budget-impact associated with the formulary decision) could collectively enhance payer' EDM and moral behavior, further strengthening ethical formulary decisions.

Select and Train Staff and Standardize Process

- Formulary committee process and procedures need standardization, to introduce consistency in decision-making process, strengthen the robustness of the decisions, and improve the ability to replicate the process across regional health insurance plans, where applicable. This is achieved by creating and documenting formulary committee SOPs and training the committee members and support staff on the SOPs.
- Identifying diverse and qualified HCPs with various RD knowledge so that they can be called upon to serve in formulary committee based on committee agenda and RD condition under deliberation, could not only enrich the functioning and robustness of formulary deliberations, but also help with the recognition of true unmet need of RD patients and the moral issues associated with including or non-including the RD drug in the formulary, setting a strong foundation for formulary committee deliberations.
- Additional training of the committee members on evidence synthesis and drug value assessment will strengthen the robustness of decision-making process and improve outcomes.

Engage in Transparent Stakeholder Communication

- Complementing the process to solicit patient stakeholder input, the channels by which patients/consumers could share such input can be publicized via company websites, newsletters and other consumer-facing materials, to foster payer-patient engagement.
- An outline of formulary committee composition (in terms of staff qualifications/background), standards, and procedures can be publicized in payer organization websites and highlight the drug value assessment criteria/standards. The outcome of formulary committee decisions (incl. deliberations on relevant patient input) could be publicized to depict payer's actions towards patient-centricity. All these steps could foster patient/consumer trust and loyalty and enhance payer credibility and reputation.
- Finally, establishing channels to periodically solicit external (patient/customer) stakeholder feedback on various actions/behaviors of the organization could help payers showcase their patient-centricity and improve the customer view of their CSP, reaping the downstream associated benefits.

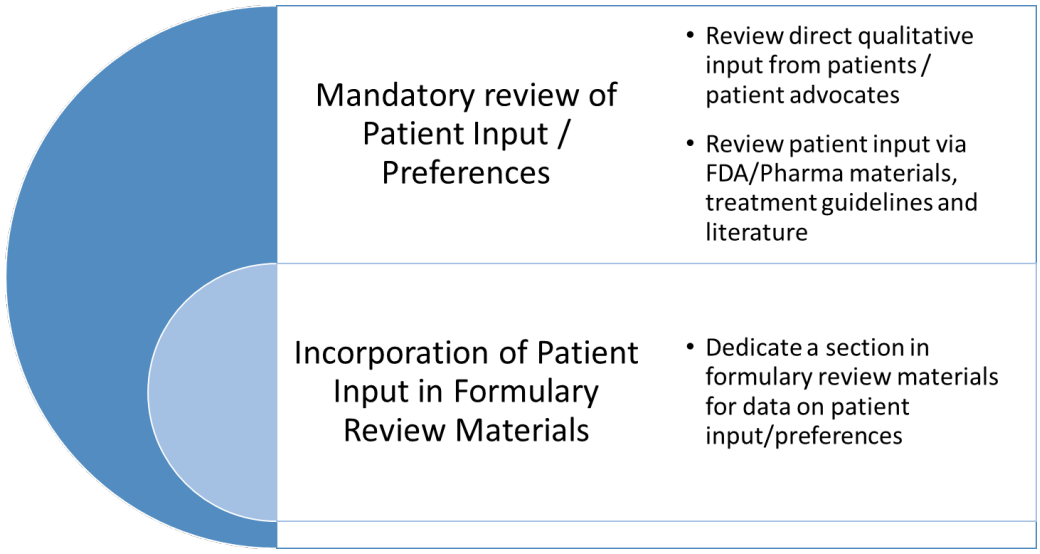
6.2 Incorporation of patient stakeholder input

Both payer and patient stakeholders identified several formal and informal channels to solicit such input. The fruit of this effort is fully realized when such input is incorporated into payer formulary decision-making process and inform final decisions, with downstream positive effects reflecting positively on payers. The two key steps payers could take to ensure patient stakeholder input is used or incorporated within the drug formulary decision-making process are outlined below, and depicted in Figure 3:

Mandatory Review of Patient Input/Preferences:

- Making the review of patient input/preferences mandatory for drug formulary committees is a critical step for payer's patient centricity and informed decision-making. Review of direct qualitative input from patients/advocates (obtained via formal or informal channels), and review of patient input via FDA/drug manufacturer materials, treatment guidelines and literature related to RD drug(s) of interest can be completed in a structured manner prior to convening the formulary committee meeting.

FIGURE 3
Incorporation of Patient Stakeholder Input



Source: Narayanan (2022)

Incorporation of Patient Input in Formulary Review Materials:

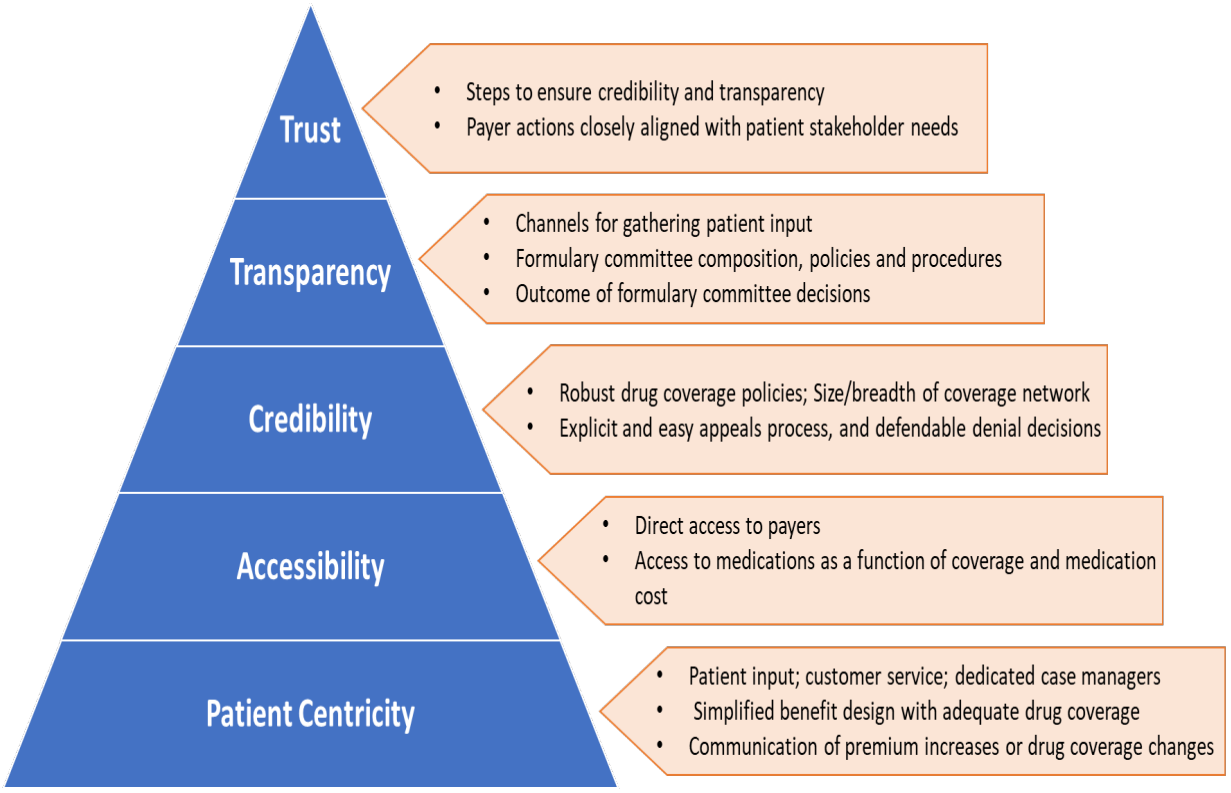
- In the formulary review materials (such as drug monographs), a section can be dedicated for review and summary of patient input/preferences related to RD condition(s) and RD drug(s) of interest. This complements the aforementioned mandatory review requirement and ensures consideration of patient input in drug formulary decision process.

6.3 Factors Influencing Stakeholder View of CSP and Corporate Reputation

Patient stakeholder's perception of payers in general and their corporate reputation was mostly negative, as revealed by this dissertation research. As a function of aforementioned payer actions concerning RD drug value assessment and stakeholder communication, and incorporation of patient input in formulary decision-making process, several organizational attributes could be positively impacted, including the customer view of CSP, their corporate reputation, customer retention, and their eventual financial performance. Patient centricity is the most foundational attribute for payer organizations, as they strive to meet the needs of patients (their customers); accessibility (to

medications and payers) and credibility were identified as key factors influencing patient perceptions and associated choice of health plans.

FIGURE 4
Factors Influencing Stakeholder View of CSP and Corporate Reputation



Source: Narayanan (2022)

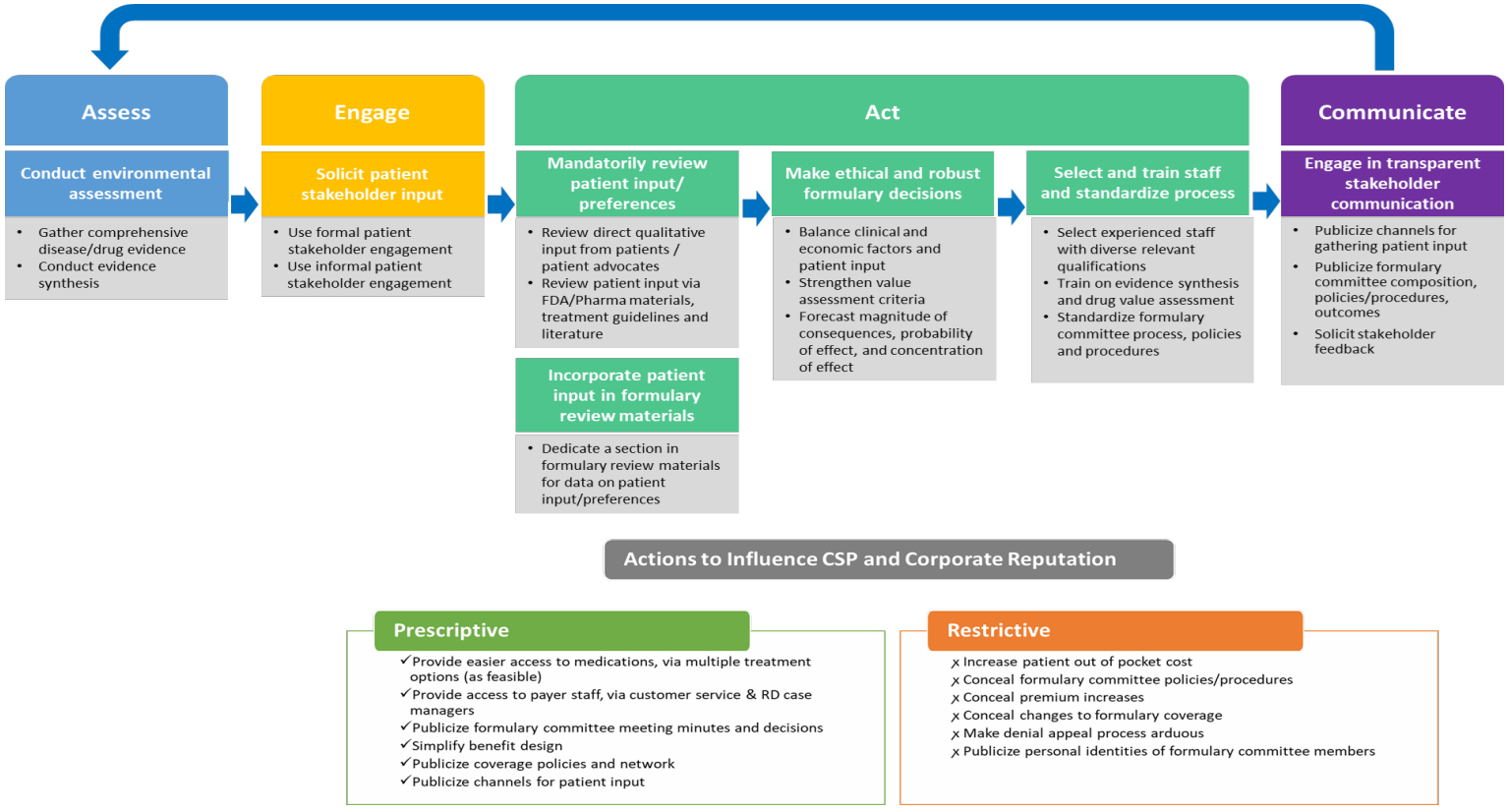
Transparency in activities and external communication are the last but key steps that payer organizations need to embed into their modus operandi, which could help payers shed their corporate for-profit persona and put a human face to their operations, building upon the previous attributes of patient-centricity, accessibility and credibility. All these actions lead to patient trust in payer organization, which in turn collectively influence patient stakeholder view of payer's CSP and corporate reputation, as depicted in Figure 4.

6.4 Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input

The integration of findings from the content analysis, the literature review of academic domains and the semi-structured qualitative interviews of payer and patient stakeholders from the fieldwork informed the praxis gap that led to the generation of the 'Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input' as illustrated in Figure 5. This framework is a function of the components described in Figure 2, Figure 3, and Figure 4. Both the literature and stakeholder interviews confirmed that there is currently no formal mechanism for payer organizations to solicit and incorporate patient stakeholder input concerning RD drug's value in their formulary decision-making process to aid informed drug coverage decisions. In the absence of prior experience, payer organizations may face a daunting task of expanding their capabilities for patient/customer outreach, refining their current modus operandi surrounding formulary decisions, including patient stakeholders and their input in their existing formulary deliberation process, and publicizing these approaches to the external audience, to derive benefits such as increased reputation and customer retention. The proposed framework in Figure 5 provides specific actionable guidance for payers on these topics.

A pragmatism of a framework relies on the ease of beginning the process that ensures success in achieving the laid-out objectives. Correspondingly, payers should begin their organizational transformation towards patient-centricity and active patient-engagement by conducting a thorough environmental assessment of evidence on RD and its treatment options and patient input/preferences documented in the literature to comprehend patient burden and unmet needs and contextualize the moral issue associated with the impending drug formulary coverage decision. Such information shall be converted to disease/drug monographs for formulary committee members to review. The next step in the framework is for payers to establish formal and informal channels for soliciting patient input, by routinely convening patient stakeholder ad boards and focus-groups on relevant RD topics as well as solicitation of written or verbal testimonials and anecdotes from patient stakeholders for relevant RD drugs. The next step is to incorporate such patient input into formulary committee review materials and make it mandatory for formulary committee members to consider and deliberate on patient input and preferences related to the RD condition or drug under review. These steps are expected to improve the comprehensiveness of evidence being considered from all sources, including patient stakeholder input, to augment drug's clinical and economic value propositions and understand its true value, from patient perspective.

FIGURE 5
Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input



Abbreviations: CSP, Corporate Social Performance; RD, Rare Disease.

Source: Narayanan (2022)

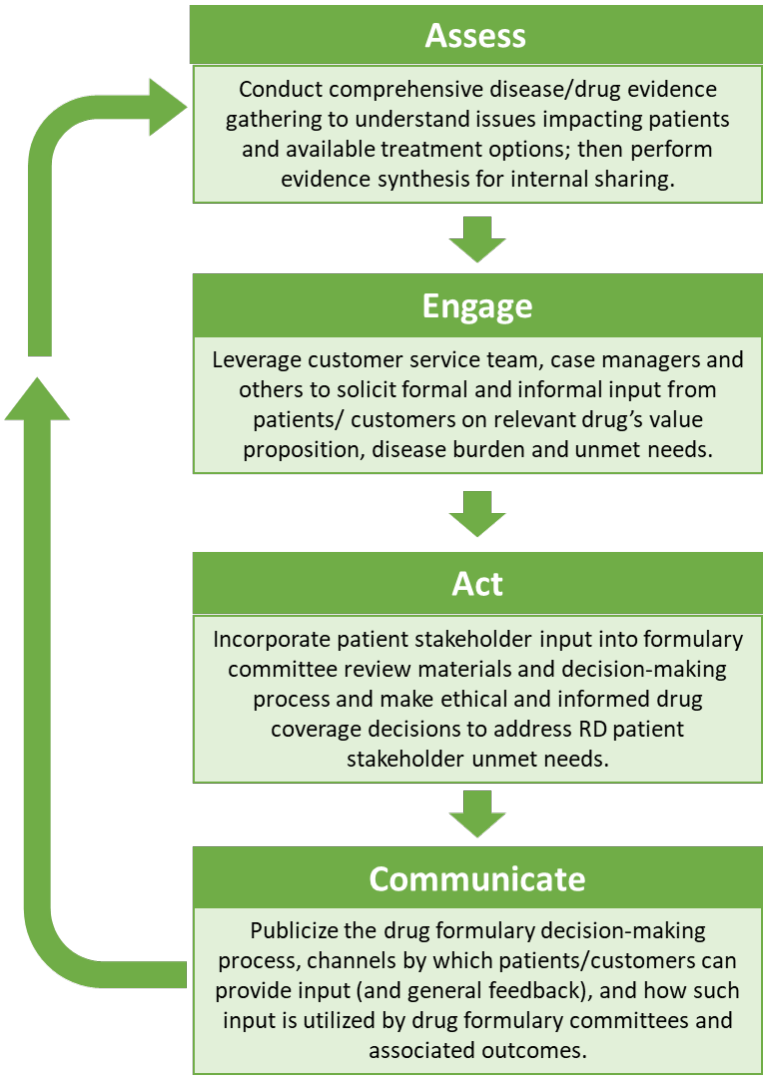
Further, such underlying comprehensive evidence would equip formulary committee members to forecast probability of positive or adverse effect of their formulary decisions, the magnitude of consequence of their actions, and the concentration of effect on the concerned RD population and their own organization's bottom-line (via drug budget-impact). These efforts collectively strengthen the robustness of formulary decision-making process and facilitate EDM. Such informed decisions may not always be in favor of drug coverage that RD patients want, but at least, such decisions appear ethical and become justifiable in the eyes of patients (payer's customers) once it is revealed that the formulary committee deliberation process was robust, encompassing consideration of patient input, and accompanied by some erudite justification of the final decision.

Standardizing the above approaches to drug value assessment and formulary decision-making, selecting staff with diverse backgrounds, and training all the staff (both committee members and support staff) on new formulary committee policies and procedures will ensure the sustainability and replicability of patient-centric and evidence-centric approaches and decisions that are bound to benefit patient stakeholders on the long-run. Externalizing the refined payer formulary committee policies and procedures, outlining the committee meeting deliberations and outcomes, and explicitly highlighting the channels by which patient stakeholders can share their input/preferences as well as their feedback on (payer) organizational corporate behaviors/actions are the final critical steps that can instil both credibility and trust in the minds of patients/customers, thereby potentially influencing the perception of CSP and increasing the payer reputation and the downstream benefits associated with it, such as customer retention and growth, potentially strengthening of organization's financial performance.

There are however specific prescriptive and restrictive actions that payers need to consider, to ensure that the framework for patient stakeholder engagement is seen in positive light and enhance patient stakeholder's view of payer's CSP and reputation. Accessibility is a key issue for patients. Payers need to provide easier access to medications - via a RD coverage policy with multiple treatment options (as feasible), and to staff - via customer service and case managers dedicated to RD areas of interest, to answer any questions about coverage policy. Simplifying benefit design that makes it easy for patients/customers to compare and choose between health plans, and publicizing the coverage policies, channels for gathering patient/customer input and formulary committee meeting minutes/decisions may all improve payer credibility/trust, improving their commitment/loyalty and view of CSP and reputation.
On the other hand, payers need to avoid arbitrarily increasing patient OOP cost or conceal their formulary committee policies and procedures and changes to premium/coverage; patients consistently identify these negative attributes, along with arduous coverage denial appeals process as issues that are causing distrust and

negative reputation. As payers strive to improve transparency in their decision-making process, revealing the identities of committee members to external public may draw unnecessary attention and cause burden on staff who are trying their best to make the right decisions for patients. Payers should continue to protect their staff identities, while training them about the new SOPs and the patient-centric approaches the organization is adopting, as part of their transformation to patient-centric organization that they aspire to be.

FIGURE 6
Process for Framework Implementation



Source: Narayanan (2022)

A successful implementation of the proposed framework and its sustainable adoption over time entails a continuous process that begins with an environmental assessment of RD/drug evidence, followed by patient stakeholder engagement and solicitation of input concerning drug's value, and payer actions to incorporate patient input and enhance their formulary decisions, and culminating in the external communication of the process and associated outcomes; and this process repeats again during every formulary decision-making cycle, as depicted in Figure 6.

A major focus of the framework is pragmatism, and hence the ability for any payer organization to adapt the framework to develop payer-patient engagement plan, refine their formulary decision-making process, and depict patient centricity. While the focus of the framework is RD, this can be adapted for chronic disease conditions as well and generalized for broader implementation and standardization within a given payer organization and across the payer/health insurance industry.

6.5 Contribution To Payer-Patient Engagement Literature

Patient engagement has increasingly become a topic of interest among payer organizations in the U.S. The public payers (such as Medicare and Medicaid) require HCPs to embrace patient engagement that include allowing patient access to their health data, dissemination of education materials to patients, direct communication with patients (for any reason), and/or collection of health data from patients, and these activities are tied to value-based payments made to HCPs (Heath, 2017). In contrast, the nature of private payers' patient-engagement has been direct, albeit, focused on patient wellness. Private payers aim to improve patient screening for specific health markers (such as blood pressure, cholesterol or blood glucose levels), reduce patient weight/BMI, improve patient medication adherence, improve patient health outcomes and reduce healthcare utilization (thereby, reduce payer budget impact) (Perna, 2012). Some private payers focus on price transparency and clarity about cost-sharing, as the OOP cost concerns influence patient/consumer choice of payers/health plans (Heath, 2017). Some state health departments offer ombudsman services that allow patients to provide testimonials and appeal a rejection in healthcare coverage/reimbursement with any private or public payer entities (Anderlik, 1999).

There is however no mechanism for payer-patient engagement that focuses on solicitation of patient input/preferences to inform payer drug formulary decisions. Further, payers do not have a mechanism to incorporate patient stakeholder input in a systematic manner into their formulary decision-making process. The extensive literature review and current dissertation research involving payer and patient stakeholders confirmed this status. This dissertation research helped unearth specific

mechanisms to solicit patient stakeholder input using formal mechanisms (e.g., patient ad boards, focus-groups) and informal mechanisms (e.g., written or verbal testimonials and anecdotes); patient advocacy groups were identified as key patient stakeholders to engage, where feasible. Externalization of the aforementioned payer approaches through concerted communication strategy is expected to improve payer organization's process of social responsiveness, incl. transparency and stakeholder engagement, which in turn could increase patient trust in payer organizations and improve payer reputation.

This dissertation research further helped inform the mechanism by which payers could incorporate patient input into their formulary decision-making process, via mandatory review of patient input by the formulary committee and incorporation of patient input into formulary committee review materials, using a section dedicated to patient input, enabling the formulary committee to comprehensively consider all evidence and patient unmet needs, recognize the moral issue in hand, and undertake the moral behavior/action of ethical and robust drug formulary decisions. Correspondingly, standardization of drug value assessment steps, formulary committee deliberations and decision-making process could improve payer's patient-centric and evidence-centric approaches and help payers derive optimal benefits from direct patient engagement, while benefiting patients on the long-run via improved access to drugs of high value. Finally, the dissertation research revealed the potential for payers to improve their customer's view of their CSP and corporate reputation via concerted patient-centric and patient engagement strategies that focuses on the facets of patient-centricity, accessibility, credibility, transparency and trust. These findings collectively fill a critical gap in existing literature on these important topics.

The final proposed 'Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input' was postulated upon these pillars of environmental assessment (starting with evaluation of disease/drug evidence from all relevant sources), patient engagement (that centers around the solicitation of patient input), payer action (that leverages patient input to improve drug value assessment and formulary decisions) and payer communication (about their intentions, actions and outcomes), laying the foundation for its practical application in routine business practice within private payer organizations in the U.S. This framework is the most critical contribution to the payer-patient engagement literature. An informed and ethical (payer) decision-making that benefits multiple stakeholders in a responsible manner while maintaining the sustainability of the healthcare ecosystem is bound to have positive impact on the U.S. healthcare sector.

7. Recommendations For Further Research

The present research was carried out to examine the characteristics of a new Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input, built upon the foundations of patient-centricity and EDM to improve patient/customer access to needed medicines and improve health outcomes. The researcher recommends that the outcome of this research could be applied as a basis for future research within the domains of payer-patient engagement, drug value assessment, and collaborative multi-stakeholder decision-making studies in the context of RD and chronic diseases.

1. Testing the newly developed framework by considering the complexities of collaborative multi-stakeholder decision-making when there is no precedence to such approach among the U.S payer organizations. The components as presented in the contextual presentation of the influence on people in individual payer organizations and the broader health-insurance industry need to be further investigated. Guidelines to prioritize strategies for implementation of the components can be developed to assist payer and patient stakeholders further.
2. Extending the level of analysis beyond the micro and meso levels by including the macro level. It is recommended for future research to extend to the macro level to include government, national policy makers and regulatory bodies informed by the identification of the main stakeholders influencing the drug value assessment and access to medications within the U.S healthcare ecosystem.
3. Testing the framework in the context of payer drug value assessment for chronic disease conditions and consideration of patient input in that decision-making process, to unearth additional nuances to payer dynamics. Such research could add value to the current framework built upon the facets of RD and in turn enrich the broader application of this framework in other areas such as non-medicinal interventions, incl. medical devices, surgery and healthcare services meant to improve patient health outcomes.
4. Replication of the present research among non-traditional payers, such as accountable care organizations (ACOs) in the U.S. ACOs increasingly assume risk, directly and indirectly influence the quality and coordination of health care services for a population of patients, and pay HCPs based on quality and value of care delivered. Incorporation of patient input/preferences in ACO decision-making process could add value to ACO's goal of improving population/patient health outcomes, and hence warrant scrutiny.
5. Replication of the present research using the qualitative approach followed by the quantitative method to further quantify the significance and importance of the resultant findings in payer-patient engagement and other applied research

arenas. A broader and diverse sample of patients/consumers and payers from across the U.S could be engaged in a quantitative survey initiative to assess specific aspects of the proposed framework to reduce impediments to behavioral and operational changes that could enhance payer-patient engagement at a larger scale and make the consideration of patient stakeholder input/preferences a norm in collaborative payer EDM regarding access to medicines in the U.S healthcare settings.

The aforementioned points represent critical and salient issues exposed by the dissertation research that should be the focus of future investigations.

8. Conclusion

The Framework for Payer-Patient Engagement and Payer Incorporation of Patient Input fills a critical gap in the literature and provides empirical guidance for payer organizations to: (1) solicit patient stakeholder input using formal mechanisms (e.g., patient ad boards, focus-groups) and informal mechanisms (e.g., written or verbal testimonials and anecdotes); (2) incorporate patient input into their formulary decision-making process (via mandatory review of patient input by the formulary committee and incorporate of patient input into formulary committee review materials, using a section dedicated to patient input); (3) externalize these patient-centric approaches through concerted communication strategy, improving transparency. The researcher recommends that the outcome of this research could be applied as a basis for future research to develop guidelines to prioritize strategies for implementation of the components (of the proposed framework) to assist payer and patient stakeholders to absorb the framework better and explore the application of framework in other relevant areas such as non-medical healthcare interventions, and non-traditional payer settings.

Keeping in mind the noted conclusions and recommendations it is anticipated that payer-patient engagement may develop further into its own unique domain of study. The isolated and identified characteristics that are unique to payer-patient engagement and payer's multi-stakeholder collaborative and informed EDM process have the potential to reinvigorate the mainstream dialogue on patient centricity of payers and EDM within payer organizations in the U.S. If successful, this aspect may be the most important contribution that the study of payer-patient engagement and payer consideration of patient input may in turn give back to the academic and professional communities. Further, the guidelines of the framework could assist payer organizations to improve their process of social responsiveness (incl. issue and stakeholder management) and patient engagement and shed negative public perception of corporate bureaucracy and greed and improve their customer's view of CSP and their organization's reputation.

The framework may offer venues for patient advocacy groups to channel their input to payers to influence medicine coverage decisions that matters the most to their constituents (i.e., patients/caregivers). By virtue of undertaking the suggested approach to soliciting and considering patient input/preferences to augment drug value assessment in formulary deliberations, the framework could offer the opportunity for payers and health policy researchers to enhance existing drug value assessment frameworks (VAFs), thereby improving healthcare delivery and population outcomes.

Payer drug formulary decisions informed by patient stakeholder input may improve drug coverage, aligned with patient stakeholder desire/interest. However, coverage does not simply relate to access to drugs and services; It implies ample, quality services that all patients who need them are able to reach and use when they need them (CSDH, 2008). One would hope that the proposed payer-patient engagement framework informed by this dissertation research is just a starting point for payer organizations to build a robust strategy to engage their constituents, enhance healthcare experience and achieve an equitable and sustainable healthcare ecosystem that benefits all actors involved.

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List of Abbreviations

ACA	Affordable Care Act
ACC/AHA	American College of Cardiology/American Heart Association
AE	Adverse Event
ASCO	American Society of Clinical Oncology
BMI	Body Mass Index
CADTH	Canadian Agency for Drugs and Technologies in Health
CSP	Corporate Social Performance
CSR	Corporate Social Responsibility
EDM	Ethical Decision Making
FDA	Food and Drug Administration
HCP	Healthcare Professional
HMO	Health Maintenance Organization
HTA	Health Technology Assessment
ICER	Institute for Clinical and Economic Review
MCO	Managed Care Organization
MSKCC	Memorial Sloan Kettering Cancer Center
NCCN	National Comprehensive Cancer Network
NHB	Net Health Benefit
NHS	National Health Service
NICE	National Institute for Health and Care Excellence
P&T	Pharmacy and Therapeutics
PAG	Patient Advocacy Group
PBM	Pharmacy Benefit Management
PCORI	Patient-Centered Outcomes Research Institute
PPVF	Patient-Perceived Value Framework
PRO	Patient Reported Outcome
OOP	Out of Pocket
QOL	Quality of Life
RCT	Randomized Controlled Trial
RD	Rare Disease
U.K.	United Kingdom
U.S.	United States
VAF	Value Assessment Framework
WHO	World Health Organization