

Value-Based Management of Specialty Drugs: Practical Considerations and Implications for Pharmacy

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The United States has long struggled to craft health care policy that balances cost, quality, and access.¹ When HHS, the primary arbiter of health care policy at the national level, issued its strategic plan for 2018-2022, its first objective was system reform, motivated by continued increases in health care spending.² Prescription drugs are among the fastest growing drivers of such spending.³ The effect is greatest among branded specialty pharmacy (SP) drugs, which account for 46.5% of spending on pharmaceutical products, despite comprising only 1.9% of prescriptions dispensed.⁴

These characteristics make SP drugs attractive targets for more active management, and they are pertinent to 3 of the policy objectives promulgated by HHS: (1) Promote affordable health care while balancing spending on premiums, deductibles, and out-of-pocket costs; (2) expand safe, high-quality health care options and encourage innovation and competition; and (3) improve Americans' access to health care and expand choices of care and service options.

Achieving these aims depends not only on government programs but also on health insurance businesses, which must execute on them while remaining competitive in their respective markets. Value-based management of health benefits is increasingly seen by both policy makers and health plans as the most promising approach to this area of reform, largely because it promises to reward outcomes instead of a drug's price or sales volume.

Proposed approaches for achieving this aim thus depart from the traditional contracting model in which health plans receive rebates from pharmaceutical manufacturers in exchange for favorable formulary position, less stringent utilization management (UM) criteria, or volume targets (see [Table 1](#)). Most prominent among them are value-based pricing (VBP), value-based insurance design (VBID), long-term financing (LTF), and outcomes-based contracts (OBCs). However, the degrees to which they are suitable for furthering HHS' aims vary.^{5,6}

VBP approaches, which hold the greatest promise, estimate benchmark prices via transparent and replicable analyses of outcomes data. Depending on the expected budget impact and rarity of disease, the acceptable value-based price point may fall above or below this analytic benchmark.⁶ Drugs meeting this threshold are offered to

ABSTRACT

OBJECTIVES: Concerns about high and rising drug prices have prompted a call to manage prescription drugs according to their value. Although not all proposals referred to as "value based" are well suited to advance this mission, health plans must select among them under the influence of competing demands and constraints of their market and nonmarket environments. To understand the implications for health policy, we sought to explore how health plans might select among and implement these approaches for specialty pharmacy (SP) under the incentives and barriers that these conditions create.

STUDY DESIGN: An experienced research team conducted a qualitative study with Blue Cross Blue Shield health plans interested in implementing value-based SP management.

METHODS: Plans' objectives, operational strategies, and factors influencing their ability to execute on these strategies were elicited in 3 focus groups.

RESULTS: Four business objectives were identified, centering on spending levels, spending variability, access to new treatments, and evidence generation for new treatments. Supporting operational strategies included increased utilization management (UM), provider and patient engagement, expanded data analytics, and adjustments to staffing models. Factors that influence their ability to act on these strategies include regional and national scale, strength of provider network relationships, disease management capabilities, business and data silos, and potential legislative actions to limit UM.

CONCLUSIONS: Health plans' preferences for different forms of SP management may not be aligned with policy objectives, particularly those that advance innovation. Policy makers should consider market and nonmarket factors that influence these preferences, including the need to mitigate spending variability and generate evidence to guide coverage decisions.

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TAKEAWAY POINTS

- ▶ Policy makers and health plans share motivations to manage specialty drugs based on their value, but not all approaches are equally suited to achieving these goals.
- ▶ Health plans experience pressure to pursue long-term financing and outcomes-based contracting approaches due to spending variability, budget constraints, insufficient clinical evidence, competitive pressures, and data and human resource constraints.
- ▶ Advancing value-based management of specialty prescription drugs will require policy initiatives that incentivize value-based pricing and insurance design.

TABLE 1. Approaches to Managing SP Drugs

Concept	Definition
Traditional contract	A contract between a drug's manufacturer and a health plan in which the manufacturer commits to paying a rebate to the health plan in exchange for favorable tier placement and UM criteria or specific volume targets
Value-based pricing	A manufacturer prices its drug according to the magnitude of its benefit, and the health plan gives it favorable tier placement and UM criteria.
Value-based insurance design	A health plan reduces a patient's out-of-pocket expenses for a drug that has demonstrated superior outcomes or value over other therapeutic alternatives.
Long-term financing	A health plan pays for an expensive drug in installments over time instead of at once.
Outcomes-based contract	A contract between a drug's manufacturer and a health plan in which the manufacturer commits to paying a refund or rebate to the health plan if plan beneficiaries who have been treated with the drug do not experience the agreed-upon outcomes

SP, specialty pharmacy; UM, utilization management.

patients without encumbrances such as high out-of-pocket (OOP) costs and aggressive UM.

VBP involves the latter part of VBP—reducing or eliminating patients' OOP costs for treatments with outcomes or value superior to those of other therapeutic alternatives.

In LTF, expensive drugs are paid for in installments, whereas OBCs offer refunds should patients not experience expected outcomes. Both approaches lack analytic benchmarks or management based on value of outcomes.

Although policy makers and health plans may agree in principle on the aims of value-based management, their expectations for execution may be different. Unlike accountable care and pay-for-performance models, for which CMS demonstration programs collect data, operationalizing and assessing value-based SP management has been left largely to commercial health plans. These companies, each with market shares of 15% or less,⁷ administer benefits for a wide range of customers, including individuals, employers, Medicaid, and Medicare.⁸ This fragmentation limits systematic understanding of conditions favoring the adoption of one model over others, and it limits plans' abilities to meet both policy and business objectives.

This study thus investigated plans' business objectives and operational strategies for managing SP drugs, focusing on factors that influence the uptake of different approaches.

METHODS

To explore the management of SP drugs relative to evidence of their value, the Drug Pricing Lab at Memorial Sloan Kettering Cancer Center (MSK) and the Blue Cross Blue Shield Association (BCBSA) conducted a qualitative study with Blue Cross Blue Shield (BCBS) health plans. Structured focus groups explored practical concerns driving decision-making around cost, quality, and value.

Focus Groups With Plan Pharmacy Executives

Pharmacy executives, including chief pharmacy officers, vice presidents, and directors of pharmacy of individual health plans, were invited to participate as representatives of BCBSA's 36 member plans (1 representative per plan). A plan was considered included in the study if a representative participated in any of the focus groups. Participants were familiar with the study, as it was part of a broader BCBSA effort to develop and implement an in-house systematic comparative effectiveness model for SP. Compensation was not offered. MSK's

institutional review board exempted this study from review.

First, using discussion guides (see [eAppendix A](#) [eAppendices available at [ajmc.com](#)]) developed by the research team, a trained moderator (A.K.) conducted 3 focus groups, on the topics of cost, quality, and value of SP projects, respectively. Each 90-minute session started by introducing the topic and then elicited plans' business objectives and operational strategies with respect to the topic. Each plan was represented by 1 individual, with the exception of 1 plan, for which a delegate stood in for 1 focus group. All groups were conducted via teleconference. Data were collected during the teleconferences by researchers in MSK and BCBSA offices where the calls were held.

Notes transcribed by 2 authors (J.O., J.K.) and 1 individual mentioned in the Acknowledgments (B.M.) were adjudicated (J.O.) and reviewed for accuracy (A.K., R.M.). A codebook was developed (J.O., B.M.) to identify and analyze emergent themes. From these themes, commonalities were identified and categorized according to whether they were business objectives, operational strategies, or factors influencing plans' abilities to implement these operational strategies (see [eAppendix B](#)). To ensure fidelity, 3 follow-up interviews were conducted with 4 plans, and preliminary findings were circulated among participants.

Plans were selected for follow-up interviews based on size, regional dominance, and influence over their provider networks.

Focus groups were conducted between February 21, 2018, and March 8, 2018, and follow-up interviews were conducted between April 12, 2018, and June 13, 2018. Thematic abstraction was completed by July 2018, with preliminary findings sent to participants in July 2018 (see [eAppendix C](#) for CoreQ checklist).

RESULTS

Of the 36 invited plans, 22 participated in 1 or more of the 3 focus groups. Plans have multiple lines of business, which represent contracts with certain types of clients, including commercial entities, Medicare, Medicaid, and individual plans through Affordable Care Act state exchanges. Collectively, participating plans cover more than 34 million beneficiaries, with most (77%) under contracts with commercial entities (see [Table 2](#)).

Responses from focus group participants could be clustered by 53 themes ([eAppendix B](#)), from which 4 business objectives and 5 operational strategies were identified.

Business Objectives

First, to meet customers' preferences, participants sought to (1) make new treatments available to patients and employers. However, they also reported that high prices and expanding indications for SP drugs increase spending variability, putting pressure on budgets and premiums. Further, although customers seek access to new treatments, they are increasingly sensitive to their prices. Thus, plans also aim to (2) control rising drug costs for plans, patients, and employers and (3) reduce spending variability. Historically, plans have used evidence-based coverage determinations to strike a balance between ensuring that patients benefit from new treatments and controlling costs and variance. Increasing FDA approvals based on early-phase data and surrogate end points motivate plans to (4) compensate for reduced clinical evidence levels.⁹

Operational Strategies

Participants identified 5 operational strategies to support their objectives. The first was to (1) intensify UM, tightening control over new treatments with uncertain clinical value and high potential budget impact. To that end, plans must determine what steps are warranted under their specific circumstances to strengthen existing UM tools, such as prior authorization, step edits, and reauthorization requirements.

Plans also aim to (2) increase engagement with patients, providing more information about treatments and encouraging them to

adhere to, switch, or discontinue treatments depending on expected outcomes. This means preparing patients for high OOP costs and potentially offering benefit designs that reward selecting higher-value treatments over lower-value options. Additionally, plans aim to (3) increase engagement with providers through risk-sharing contracts and education about SP management and costs.

To reduce uncertainty about outcomes of new treatments, participants also reported ambitions to (4) expand data and analytics capacities. They particularly seek to combine real-world data from beneficiaries—including electronic health records (EHRs), pharmacy and medical claims data, and patient surveys and monitoring—to assess treatment patterns, adherence, and outcomes and to identify patients at risk of requiring high-cost interventions.¹⁰

Finally, plans reported (5) adjusting staffing models. FDA approvals with low-level evidence limit their ability to craft utilization criteria, calculate and share risk with providers and manufacturers, and offer accurate treatment information to patients and clinicians. Further, pharmacy staff report being overwhelmed by the volume of assessments. Many plans thus aim to hire more pharmacists, statisticians, and data scientists. They also aim to restructure existing programs and bridge operational and data silos—for example, by expanding the scope of disease-specific management programs and tightening connections between contracting and pharmacy staff.

TABLE 2. Health Plan Participation and Characteristics (fall 2019)

Participation	N (plans)
Number of plans invited to participate in focus groups	36
Number of plans participating in any focus group ^a	22
Number of plans participating in each focus group, by topic	
Cost	18
Quality	14
Value	8
Characteristics	
Regional location of plans ^b	
Northeast	5
Midwest	6
South	7
West	4
	N (plan beneficiaries)
Total number of beneficiaries (n=22 plans)	40,134,244
Mean (SD)	1,824,283 (1,616,907)
Median (IQR)	1,013,360 (554,361-3,018,683)
Total number of beneficiaries by line of business	
Commercial (including Federal Employee Program)	26,378,026
Medicare (MAPD, Part D Prescription Drug Plan)	2,928,885
Individual	3,586,776
Medicaid	1,406,253

IQR, interquartile range; MAPD, Medicare Advantage and Part D prescription drug plan.

^aPlans could participate in more than 1 focus group.

^bClassified according to headquarters location.

Factors Affecting Implementation of Operational Strategies

Participants identified several factors that affect the extent to which plans can enact these strategies. For example, opportunities to engage with providers vary substantially. Plans that own practices or are the dominant insurer in their region report some success in influencing treatment selection through clinician education and risk-sharing arrangements. In contrast, plans with lower regional patient share and more distant provider relationships struggle to exert influence. Notably, both large national and small regional plans reported this constraint.

Patient engagement strategies are similarly challenging. Historically, disease management programs have initiated contact and offered patients guidance. However, their disease-specific structure limits scaling them to broader populations, and their capacity to monitor adherence and outcomes is limited.

Business and data silos pose additional barriers. For example, opportunities to integrate pharmacy and medical claims are often limited, because plans either cannot access data from their pharmacy benefit manager (PBM) or lack organizational capacity for data integration and analytics. Addressing this issue may require restructuring PBM relationships or departing from them at the expense of rebate revenues. This also holds true for plans with members who are receiving pharmacy benefits through separate PBM-employer contracts.

UM intensification efforts also face barriers. For instance, manufacturers offer patients co-pay coupons to defray OOP expenses and programs for navigating prior authorization requirements. Participants also voiced concerns that state-level right-to-try legislation would result in patients receiving unproven investigational treatments, leaving them to contend with poorly understood treatment outcomes and adverse events (see [eAppendix Table](#)).

DISCUSSION

The business objectives and operational strategies described by health plans in our study suggest that value-based management of SP drugs is desirable but difficult to implement.

Alignment of Health Plan Business Objectives and Operational Strategies With HHS Policy Aims

Their business objectives suggest that health plans share several ambitions expressed in HHS policy aims, including balancing costs with access to new treatments. However, there is a disconnect between the HHS aim to foster innovation and plans' objective of reducing spending variability, because many of the newest drugs on the market are intended for use by small patient populations but at high prices. Further, HHS aims do not include plans' objective of compensating for reduced evidence levels. Although evidence of benefits and harms is at the heart of value-based management, it is unclear how HHS views its role relative to its aims. For health plans, however, the lack of available evidence is seen as a barrier

to crafting informed coverage policy, and developing their own evidence is the closest solution at hand.

This discordance between policy aims and business objectives suggests that the SP management approaches preferred by individual health plans may be driven by considerations that are not appreciated or shared by federal policy makers.

Spending Levels and Variability

Spending variability associated with high-cost treatments for a small number of patients is a primary concern for all plans. LTF, which distributes spending over time, is the only one of the SP approaches to address this directly. OBCs may also give plans the option to negotiate terms that address some elements of uncertainty. By contrast, VBID and VBP require that even expensive treatments be made accessible to patients if prices align with value. Higher utilization of more expensive options may raise costs overall,⁹ even as it could also encourage manufacturers to offer price concessions. On the other hand, plans could also view LTF and OBCs as driving cost increases due to price inflation, as they offer no mechanism to tie management directly to value. A plan's choice in approach is likely to be informed by its ability to absorb short-term spending variability and capture long-term returns from value-based strategies.

Data Analytics

Plans are similarly constrained in their ability to expand data analytics capabilities to generate evidence. Few are equipped to engage patients who are at risk of needing high-cost treatment and then to monitor for outcomes; those plans with existing disease management programs face the prospect of a major change management effort. They must also be able to access and analyze data identifying the patients they intend to engage. Even under optimal circumstances—access to EHR, medical, pharmacy, and laboratory claims across all covered lives, as well as the ability to link these data together—the technical challenges of identifying high-risk patients and monitoring outcomes are daunting, particularly for new treatments.

Health plans that can expect success with this strategy will likely be those with the size, regional dominance, and resources to integrate and monitor data across beneficiaries. This would lend itself well to generating evidence of value needed for VBID and VBP. Conversely, smaller plans with less scale and access to data may find VBID and VBP less feasible. Although OBCs offer no assurances, such plans may view them as a way to generate data to assess outcomes that would otherwise be out of reach. Absent other sources of evidence to inform coverage policy, data integration capabilities may also influence their preferred approaches.

UM

The inability to decline coverage for certain classes of drugs, often due to federal or state laws, compels plans to mitigate the budget impact of high-priced specialty drugs by managing utilization. This is reflected in individual plans' strategies for building up their prior

authorization and step edit capabilities. This creates negotiating leverage under existing contracting models, but implications for different SP management approaches are less clear. Using UM to restrict access to high-value treatments that would otherwise be preferred under VBP and VBID would run counter to these efforts, but using it to give preference to these treatments over lower-value options supports their aim. Plans with limited opportunity to increase UM may find LTF and OBCs appealing if they promise some control over access and information. Overall, plans with the ability to increase UM are in a better position relative to their peers to engage in VBP and VBID, but they also have the negotiating leverage to successfully continue traditional contracting.

Provider and Patient Engagement

All SP approaches require communication with providers and patients about treatment selection. Although many plans seek to build engagement, opportunities vary by provider network relationships, regional dominance, and disease management program capabilities. Even plans that are able to build a regional SP management program may be unable to scale the same effort nationally. This obstacle is particularly relevant for VBID and VBP, which require open communication about which treatment options are high value and therefore preferred. Several OBC and LTF offerings aim to solve this problem through contracts and programs that avoid intensive physician and patient engagement, or step in to relieve plans of this role.⁸ Thus plans with weaker provider network relationships and fewer resources to engage patients could prefer OBCs or LTF.

Implications for HHS Policy Aims

Understanding the appeal of different SP management approaches to health plans is important for the realization of HHS policy aims. LTF, OBCs, VBID, and VBP vary in their ability to advance these aims and in their appeal to health plans. By rewarding evidence of value, VBID and VBP create clear signals to innovators developing new treatments. Because they require evidence of value in exchange for patient access, systematic use and enforcement of such policies should also spur manufacturers to provide more evidence about treatment outcomes.

Although neither HHS nor health plan cost-control objectives are advanced by LTF or OBCs, they may appeal to health plans. They are less likely than VBID or VBP to run afoul of the challenges that fetter efforts to improve provider and patient engagement or data analytics. They also appeal to plans' desire to mitigate spending variability—a rising priority as increasingly expensive treatments enter the market. By comparison, managing access to SP drugs according to value could require plans to forgo rebate revenues from lower-value treatment options, putting them at a competitive disadvantage.

Limitations

Because our study included only health plans associated with BCBS, our findings may not generalize to other health plans. However, the health plans in our research hold more than 35 million covered lives,

representing 12% of the US market.¹¹ Our effort was also limited in scope to traditional specialty drugs. Our findings therefore may not necessarily apply to other product categories. However, strategies to manage SP drugs according to value may generate broader lessons, as well as positive spillover effects for other areas of health care, such as better coordination between providers and payers or technological innovations that facilitate monitoring of patient outcomes regardless of treatment.

CONCLUSIONS

Policy makers seek to make new treatments available while balancing cost concerns. Although health plans share this aim, they also seek to mitigate spending variability and compensate for the low levels of evidence for new, high-cost SP drugs entering the market. The latter considerations drive preferences toward LTF and OBCs for many plans, which are inferior to VBP and VBID for meeting policy aims to advance innovation. Plans that can expect a positive impact on their business from VBP and VBID are generally those with stronger relationships with provider networks, greater ability to engage patients and manage utilization, and sufficiently large size to absorb short-term spending variations. However, these characteristics also give them the ability to maintain traditional contracting strategies, which have historically perpetuated the delinkage of SP drug prices from their value.

Policy makers who are seeking to advance value-based management of SP drugs should consider reforms that address both limiters and incentives in the market and nonmarket environments in which plans operate. For example, policies and demonstration programs that increase negotiating leverage and encourage management of drugs according to their value could offer a starting point for aligning incentives with VBP and VBID. Others, such as requiring that rebates be passed through at the point of sale, could reduce the appeal of more traditional contracting models while also benefiting patients. Finally, more stringent evidence requirements for FDA approvals and enforcement of postmarketing commitments for drugs with accelerated approval would provide more information for value-based management. ■

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manuscript (JK, SC, JC, RM, AK); critical revision of the manuscript for important intellectual content (JK, SC, RM, AK); statistical analysis (AK); provision of patients or study materials (AK); obtaining funding (AK); administrative, technical, or logistic support (JK, JO, JC, AK); and supervision (JK, JC, RM, AK).

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eAppendix A. Focus Group Discussion Guides

Focus Group on Cost

Introduction and Overview

- Thank you for participation
- Brief introduction to identify moderator (AK) and note taker (JO, JK, BM)
- Describe objectives for today's focus group:
 - Help us get a comprehensive understanding of concerns about cost across your plans
 - Identify internal and external factors that shape Plans' abilities to respond to these concerns
- Review structure of discussion
 - A brief roll call and introduction – 15 minutes
 - A discussion to understand your specialty pharmacy cost concerns – 20 minutes

Identifying what your objectives are in addressing these issues – 20 minutes

- Characterizing key internal and external factors that affect your ability to respond – 20 minutes
- A brief verbal summary of key themes of the discussion – 5 minutes
- Lay out ground rules / set expectations for moderation that allows AK to actively manage discussion
 - Are hoping for interactive discussion
 - Limited time to capture insight from a large group
 - Be prepared for AK to actively manage the discussion, which may include tabling some topics for further discussion, and guiding conversation to certain participants as needed

Brief roll call and introduction

- To match voices and names of the participants:
 - Please introduce yourself and provide a quick overview of your plan
 - Tell us why you are joining today's focus group on cost

Understanding key specialty pharmacy cost concerns

- Thinking about your business overall, what costs, current or future, keep you up at night?* Where does pharmacy cost rank among these concerns? *Probe for:*

- Ability to contain costs
- Ability to measure/understand costs
- Ability to incorporate pharmacy costs, medical cost offsets and other, related information into formulary decision-making
- What are the concerns among your members about specialty pharmacy and costs?

Identifying objectives in addressing specialty pharmacy costs

- Ask each participant to spend 1 minute or less summarizing the key points in your notes
- Cross check similarities and differences across plans

Characterizing internal and external factors that affect ability to respond

- Use pre-work exercise responses to segue into internal and external factors.
- Today and in the coming 5 years
 - What barriers would keep you from achieving your aims?
 - What factors will be necessary to achieve your aims?*
- *Probe for internal and external barriers and factors*:*
 - Policies
 - Resources
 - Time
 - Data
 - Money
 - People
 - Capabilities
 - Analytics
 - Actuarial
 - Other?
 - Ability to understand and navigate
 - Definitions and common terminology
 - Transparency
 - Education (particularly of leadership)
 - Ability to demonstrate ROI
 - Competition (in health insurance marketplace)
 - Certainty
 - Synergies / B2B relationships
 - Leadership – reactive vs. proactive

Wrap-up and conclusion

- Brief summary of high-level findings:
 - Key concerns about costs fall into the following groupings [x, y, z]
 - We heard the following objectives in addressing these issues are [x, y, z]
 - And the critical barriers to achieving these objectives are [x, y, z]
- Anything missing, or that anyone would add?
- Final conclusion and thank you.

Focus Group on Quality

Introduction and Overview

(See Part I. Focus Group on Cost)

Brief roll call and introduction

(See Part I. Focus Group on Cost)

Understanding key specialty pharmacy quality concerns

- In the last focus group we discussed cost; today we're discussing quality. By "quality" we mean measures of a drug's efficacy and safety. This includes evidence from clinical trials, such as primary and secondary endpoints and adverse events, as well as real world evidence on persistence and discontinuation. It can also include patient-reported outcomes and quality of life.
- Thinking about your business overall, what are your greatest areas of concern when you think about quality?* How does that differ for pharmacy and medical benefits? *Probe for:*
 - Ability to manage based on quality
 - Ability to measure/understand quality (including current levels of evidence and speed in FDA approvals)
 - Ability to incorporate quality into formulary decision-making
- What are the concerns among your members about quality?
- How does this affect your review of new retail and specialty drugs?
- Do you have concerns about measuring the quality of multi-indication drugs?

Identifying objectives

- Use the table below to help plans describe what they'd like to achieve in terms of measuring quality of drugs
 - Give plans 1 minute to consider which measures they currently take into considerations
 - *Probe on*
 - How these measures are currently incorporated into their decision-making
 - Whether there are other measures they'd really like to have

- What they'd like to be able to say about the way they use these and any other measures in 5 years
- Whether they can use their own patient population to verify RW performance of these drugs
- Cross check similarities and differences across plans

Measures in RA	Measures in MS	Other Measures
ACR improvement criteria (ACR20, ACR50, ACR70)	Kurtzke Expanded Disability Status Scale (EDSS)	Patient Reported Outcomes
Disease activity score (DAS-28)	Relapse Measures (Multiple)	Persistence
Health Assessment Questionnaire Disability Index (HAQ-DI)	MRI Outcomes	Discontinuation
Clinical Disease Activity Index (CDAI)	Adverse Events	Quality of Life (EQ5D and SF-36)
Sharp Score	Annualized Relapse Rate (ARR)	Number needed to treat (NNT)
Remission – Simplified Disease Activity Index (SDAI) ≤ 3.3	Time to 2 nd Relapse	
	MSRS-R	
VAS 0-10/100 Scale	Treatment Satisfaction Questionnaire	

Characterizing internal and external factors that affect ability to respond

- Use prior exercise responses to segue into internal and external factors.
- Today and in the coming 5 years
 - What barriers would keep you from achieving your aims?
 - What factors will be necessary to achieve your aims?*
- *Probe for internal and external barriers and factors*:*
 - Policies
 - Resources
 - Time
 - Money
 - Data
 - People

- Capabilities
 - Analytics
 - Actuarial
 - Other?
- Ability to understand and navigate
 - Definitions and common terminology
 - Transparency
 - Education (particularly of leadership)
- Ability to demonstrate ROI
- Competition (in health insurance marketplace)
- Certainty
- Synergies / B2B relationships
- Leadership – reactive vs. proactive

Probe for:

- Ability to engage with manufacturers on quality
- Manufacturer requests for outcomes-based contracting
- Challenges in measuring quality in the real world
- Need to prove ROI for the necessary actions
- Considerations that prioritize cost over clinical effectiveness / vice versa

Wrap-up and conclusion

- Brief summary of high-level findings:
 - Key concerns about quality fall into the following groupings [x, y, z]
 - We heard the following objectives in addressing these issues are [x, y, z]
 - And the critical barriers to achieving these objectives are [x, y, z]
- Anything missing, or that anyone would add?
- Thank you and provide contact information

Focus Group on Value

Introduction and Overview

(See Part I. Focus Group on Cost)

Brief roll call and introduction

(See Part I. Focus Group on Cost)

Understanding key specialty pharmacy value concerns

- In the last two focus groups we discussed cost and quality. Today's focus group will be the third and final one of the series, and will focus on value.
- Exercise to define value: What is your definition of value, as it pertains to specialty drugs*?
- Thinking about your business overall, what are your greatest concerns when you think about value*? How does that differ for pharmacy and medical benefits? *Probe for:*
 - Ability to manage based on value
 - Ability to measure/understand value
 - Ability to incorporate value into formulary decision-making
 - Ability to manage for value over the course of a patient's treatment
- What are the concerns among your members about value?
- Do you have concerns about measuring or managing the value of multi-indication drugs?

Identifying objectives

- We're going to conduct a brief exercise to help conceptualize how you approach the formulary decision-making process.
- Imagine that the FDA recently approved a new drug, Product X, for the treatment of moderate to severe plaque psoriasis. Please take a moment to review Product X's characteristics, as demonstrated in its registration trial with the FDA.

Indication	<ul style="list-style-type: none">• For the treatment of adult patients with moderate to severe plaque psoriasis.• Product X has no other indications at the moment, but has several in the pipeline
Mechanism of action	<ul style="list-style-type: none">• Human interleukin-12 antagonist

Route of administration and dose	<ul style="list-style-type: none"> 60 mg subcutaneous injection delivered in 1 mL suspension with NoPainEver™ autoinjector
Clinical effectiveness	<ul style="list-style-type: none"> 80% of patients attained PASI 75, compared with 5% on placebo 90% of patients attained PASI 50%, compared with 10% on placebo In older clinical trials, 70% and 75% of patients on Humira and Enbrel attained PASI75, respectively
Safety and tolerability	<p>Most common side effects were upper respiratory tract infections and headache. 1% of patient experienced side effects that resulted in discontinuation of the drug.</p>

- On the basis of clinical characteristics alone, how do you think you might manage this drug?*
- In addition to this clinical profile, what would you need to know about this drug to inform how you'd manage it? *Probe for:*
 - List price
 - Net price
 - Cost offset (short-term/long-term)
 - Other clinical information
 - Real world evidence
- Under what conditions would you give preferential treatment to Product X over the market incumbents, such as Enbrel and Humira?
- How would your answers change if Product X showed only comparable efficacy and safety to Enbrel and Humira?
- What would you do to ensure that use of the drug continues to be of value over the course of the patient's treatment?*
- Now imagine that it's 5 years from now. How would you like your answer to change from what you just described?

Characterizing internal and external factors that affect ability to manage based on value

- Use prior exercise responses to segue into internal and external factors.
- Today and in the coming 5 years
 - What barriers would keep you from achieving your aims?
 - What factors will be necessary to achieve your aims?
- *Probe for internal and external barriers and factors:**
 - Policies
 - Resources
 - Time
 - Data
 - Money
 - People
 - Capabilities
 - Analytics
 - Actuarial
 - Other?
 - Ability to understand and navigate
 - Definitions and common terminology
 - Transparency
 - Education (particularly of leadership)
 - Ability to demonstrate ROI
 - Competition (in health insurance marketplace)
 - Certainty
 - Synergies / B2B relationships
 - Leadership – reactive vs. proactive

Probe for:

- Ability to engage with manufacturers on value
- Manufacturer requests for outcomes-based contracting
- Challenges in measuring value in the real world
- Need to prove ROI for the necessary actions
- Considerations that prioritize cost over clinical effectiveness / vice versa

Wrap-up and conclusion

- Brief summary of high-level findings:

- Key concerns about value fall into the following groupings [x, y, z]
- We heard the following objectives in addressing these issues are [x, y, z]
- And the critical barriers to achieving these objectives are [x, y, z]
- Anything missing, or that anyone would add?
- Thank you and provide contact information

**Recorded responses were included and categorized in the codebook (eAppendix B).*

eAppendix B. Focus Group Response Codebook

Table 1. Key Themes Emerging from Cost, Quality, and Value Focus Groups

<i>Question</i>	<i>Key themes</i>
Areas of Greatest Concern Pertaining to Cost and Quality:	Pipeline pressure on budget
	Management need
	Customer and market pressure
	Plan-wide “interoperability”
	Competing concerns
	Uncertainty
	Resource need
	Ensuring quality of care with new therapies
	Managing access to treatment
	Ensuring appropriate use
	Information barriers
	Accounting for non-clinical benefits
	Patient expectations
	Failing to see expected value
Unmet need	
Internal and External Factors Affecting Decision-Making:	Internal buy-in
	Consistency in reimbursement
	Legislative and policy change
	Market power
	Efficiency
	Capability development
	Balancing quality and financial concerns
	Ability to coordinate care
	Resources
	Data availability
	Access to existing data
Ability to engage with other stakeholders	

<i>Question</i>	<i>Key themes</i>
Vision for Improved State in Specialty Pharmacy Includes the Following:	<ul style="list-style-type: none"> Improved benefit design Reduced variability in costs Controlling costs Changing policy Maintaining management levers Comprehensive approach Understanding costs Improved information Improved care management Changing financial practice Understanding risks and benefits Increased pressure on manufacturers Accountability of all stakeholders Greater efficiency in information gathering Stronger management capabilities Best practices Resources
Definition of “Value” for Specialty Drugs (SD):	<ul style="list-style-type: none"> Component related to clinical effectiveness Component related to cost effectiveness Patient input as a component Not currently considered
Factors necessary to ensure continued value:	<ul style="list-style-type: none"> Tracking capabilities Utilization management levers Biosimilar competition Reference pricing Information availability

eAppendix C. COREQ (COnsolidated criteria for REporting Qualitative research) Checklist

A checklist of items that should be included in reports of qualitative research. You must report the page number in your manuscript where you consider each of the items listed in this checklist. If you have not included this information, either revise your manuscript accordingly before submitting or note N/A.

Topic	Item No.	Guide Questions/Description	Reported on Page No.
Domain 1: Research team and reflexivity			
<i>Personal characteristics</i>			
Interviewer/facilitator	1	Which author/s conducted the interview or focus group?	6
Credentials	2	What were the researcher's credentials? E.g. PhD, MD	1
Occupation	3	What was their occupation at the time of the study?	1
Gender	4	Was the researcher male or female?	1
Experience and training	5	What experience or training did the researcher have?	6
<i>Relationship with participants</i>			
Relationship established	6	Was a relationship established prior to study commencement?	5
Participant knowledge of the interviewer	7	What did the participants know about the researcher? e.g. personal goals, reasons for doing the research	6
Interviewer characteristics	8	What characteristics were reported about the interviewer/facilitator? e.g. Bias, assumptions, reasons and interests in the research topic	6
Domain 2: Study design			
<i>Theoretical framework</i>			
Methodological orientation and Theory	9	What methodological orientation was stated to underpin the study? e.g. grounded theory, discourse analysis, ethnography, phenomenology, content analysis	6
<i>Participant selection</i>			
Sampling	10	How were participants selected? e.g. purposive, convenience, consecutive, snowball	5

Method of approach	11	How were participants approached? e.g. face-to-face, telephone, mail, email	6
Sample size	12	How many participants were in the study?	7
Non-participation	13	How many people refused to participate or dropped out? Reasons?	7
<i>Setting</i>			
Setting of data collection	14	Where was the data collected? e.g. home, clinic, workplace	6
Presence of nonparticipants	15	Was anyone else present besides the participants and researchers?	6
Description of sample	16	What are the important characteristics of the sample? e.g. demographic data, date	8
<i>Data collection</i>			
Interview guide	17	Were questions, prompts, guides provided by the authors? Was it pilot tested?	6
Repeat interviews	18	Were repeat inter views carried out? If yes, how many?	N/A
Audio/visual recording	19	Did the research use audio or visual recording to collect the data?	6
Field notes	20	Were field notes made during and/or after the interview or focus group?	6
Duration	21	What was the duration of the inter views or focus group?	6
Data saturation	22	Was data saturation discussed?	N/A
Transcripts returned	23	Were transcripts returned to participants for comment and/or correction?	6
Topic	Item No.	Guide Questions/Description	Reported on Page No.
Domain 3: analysis and findings			
<i>Data analysis</i>			
Number of data coders	24	How many data coders coded the data?	6

Description of the coding tree	25	Did authors provide a description of the coding tree?	Appendix B
Derivation of themes	26	Were themes identified in advance or derived from the data?	6
Software	27	What software, if applicable, was used to manage the data?	N/A
Participant checking	28	Did participants provide feedback on the findings?	6
<i>Reporting</i>			
Quotations presented	29	Were participant quotations presented to illustrate the themes/findings? Was each quotation identified? e.g. participant number	Appendix B
Data and findings consistent	30	Was there consistency between the data presented and the findings?	12-17, App. B
Clarity of major themes	31	Were major themes clearly presented in the findings?	12-17, App. B
Clarity of minor themes	32	Is there a description of diverse cases or discussion of minor themes?	12-17, App. B

Developed from: Tong A, Sainsbury P, Craig J. Consolidated criteria for reporting qualitative research (COREQ): a 32-item checklist for interviews and focus groups. *International Journal for Quality in Health Care*. 2007. Volume 19, Number 6: pp. 349 – 357

Once you have completed this checklist, please save a copy and upload it as part of your submission. DO NOT include this checklist as part of the main manuscript document. It must be uploaded as a separate file.