Barriers to Fair Access Assessment

Final Protocol

May 12, 2021

Institute for Clinical and Economic Review

Table of Contents

1. Executive Summary

The national debate about drug pricing has focused great attention on methods to determine whether the price of a drug is "fair" or "reasonable." A question far less examined is how to determine whether insurance coverage is providing fair access to that drug. It appears widely agreed that cost sharing and drug coverage criteria serve everyone's interest when they steer patients toward evidence-based use of treatments that achieve equal or better outcomes at lower costs. But this level of conceptual agreement does little to help advance thinking on how to assess and judge specific cost-sharing provisions and prior authorization protocols. Is it fair to have patients pay at the highest cost-sharing level when there is only a single drug available in a drug class? What are the circumstances in which step therapy is a reasonable approach to limiting coverage? When is it appropriate for the clinical criteria required for coverage to be narrower than the Food and Drug Administration (FDA) labeled indication? And for all of these questions, how should the pricing of a drug factor in to whether certain strategies to limit or steer patient access are appropriate?

To answer these questions, ICER has developed a set of appropriateness criteria for cost-sharing and for prior authorization protocols for pharmaceutical coverage as described in its white paper *Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals* published on September 28, 2020. These appropriateness criteria are based on analysis of prior policy and ethical research, with active deliberation and revision following a December 2019 ICER Policy Summit with representatives from patient groups, clinical specialty societies, private payers, and the life science industry.

An important next step is to put these criteria into action. ICER's **Barriers to Fair Access Assessment** will take the fair access criteria set and apply them to evaluate the coverage policies of 28 drugs across the largest formularies (by covered lives) of the 15 largest commercial payers in the US. Work on this project will begin during the summer of 2020 and the first report of the Barriers to Fair Access Assessment is scheduled for release in fall 2021.

The initial focus of this evaluation will be on coverage policies for drugs that have been shown in ICER reports to have average net prices among commercial US payers that fall within a reasonable cost-effectiveness range. ICER's goal in developing the fair access criteria was to provide a tool for all health care participants; in our initial Barriers to Fair Access Assessment we will apply the criteria set ourselves to evaluate the extent to which we believe current coverage policies meet key standards for appropriate design and implementation.

ICER will perform analyses of the proportion of criteria that are met across drugs, conditions, and payers. We will leverage the MMIT Analytics Market Access Database for formulary data.

To help provide important guidance on this assessment, the Barriers to Fair Access Assessment will benefit from ongoing input from a multi-stakeholder Working Group consisting of representatives from leading patient advocacy groups, clinical societies, private payers, pharmacy benefit managers, and life sciences companies. The Working Group will advise ICER on the application of the fair access criteria to coverage policies; provide insight into the patient experience with prescription drug coverage and access; and advise on important nuances in the interpretation of payer coverage policies.

2. Background

2.1 Background

ICER has developed a set of design and implementation criteria for drug prior authorization protocols in the September 28, 2020 white paper, *Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals*. These criteria are intended to represent requirements that must be met in order for the prior authorization protocol to be appropriate, or, in other words, to ensure fair access. The criteria are based on analysis of prior policy and ethical research, and have undergone active deliberation and revision following a December 2019 ICER Policy Summit with representatives from patient groups, clinical specialty societies, private payers, and the life science industry.

2.2 Objectives

The ICER Barriers to Fair Access Assessment will apply the fair access criteria set to evaluate the coverage policies of 15 of the largest private payers in the US. In this first iteration of the assessment, we will focus the evaluation on coverage policies for 28 drugs that have been the subject of ICER evidence reviews and have been determined to be priced within a reasonable cost-effectiveness range. The short-term goal of this assessment is to produce a report that evaluates the extent to which the prior authorization protocols for these fairly-priced drugs meet the fair access criteria. We envision this report as being repeated annually, with additional drugs and payers added to the evaluation. The overall objective of the assessment is to test whether the fair access criteria can help bring greater transparency to the public debates about fair insurance coverage for drugs and, in addition, promote the positive linkage of fair pricing with fair access that will advance the best interests of patients and the health system.

2.3 Research Questions

The overarching research question this project will address is whether the prior authorization policies for drugs priced within reasonable cost-effectiveness ranges meet the criteria for fair access. Within this broad research question, we will perform analyses to assess the rate of concordance of prior authorization policies with the fair access criteria. Separate analyses will be done to analyze rates of concordance by:

Individual fair access criterion
Drug
Condition
Across payers in scope
Individual payers

2.4 Timeline

The final report for this project will be released in fall 2021. The timeline leading up to the posting of the final report includes recruitment of a Working Group, notification to payers who will be included in the analysis, conducting the analysis, sharing a draft report with payers and allowing them time to provide the team with comments. A full timeline including milestone dates will be posted on ICER's website.

3. Role of the Working Group

To help provide important guidance on this project, the Barriers to Fair Access Assessment benefits from ongoing input from a multi-stakeholder Working Group consisting of representatives from leading patient advocacy groups, clinical societies, private payers, pharmacy benefit managers, and life sciences companies. The Working Group advises ICER on the application of the fair access criteria to coverage policies; provides insight into the patient experience with prescription drug coverage and access, including real-world examples; and advises on important nuances in the interpretation of payer coverage policies. The Working Group members are:

- **Cat Davis Ahmed,** MBA, Vice President of Policy and Outreach, Familial Hypercholesterolemia Foundation
- **Alan Balch,** PhD, Chief Executive Officer, Patient Advocate Foundation
- **Robert W. Dubois**, MD, PhD, Interim President and Chief Executive Officer, Chief Science Officer, National Pharmaceutical Council
- **Patrick Gleason,** PharmD, Assistant Vice President of Health Outcomes, Prime Therapeutics
- **Barbara Henry**, Manager, Clinical Pharmacy Services, Harvard Pilgrim Health Care
- **Leah Howard**, JD, Chief Operating Officer, National Psoriasis Foundation
- **Cliff Hudis,** MD, FACP, FASCO, Chief Executive Officer. American Society of Clinical Oncology
- **Anna Hyde,** Vice President of Advocacy and Access, Arthritis Foundation
- **Rebecca Kirch**, JD, Executive Vice President, National Patient Advocate Foundation
- **Eleanor Perfetto,** PhD, MS, Executive Vice President, National Health Council
- **Carl Schmid,** Executive Director, HIV+Hepatitis Policy Institute
- **Saira Sultan,** President, Connect4Strategies (representing The Haystack Project)
- **Bari Talente,** Executive Vice President, Advocacy, National Multiple Sclerosis Society
- **Douglas White,** MD, PhD, Treasurer, American College of Rheumatology

4. List of Included "Cost-effective" Drugs

As described in greater detail below, the process for the analysis will start by identifying drugs within ICER reviews that are currently priced in accordance with reasonable cost-effectiveness thresholds. These drugs will be termed the list of "cost-effective" drugs.

4.1 Initial list of drugs

Drugs eligible for consideration are those subject to a cost-effectiveness analysis in an ICER report from 2015 to 2020 and which were determined at the time of their original report to have an incremental cost-effectiveness ratio based on the WAC or net price at or below the price needed to reach $150,000 per equal value of life years gained (evLYG) or quality-adjusted life year (QALY), whichever price was higher. For these drugs we will update the ceiling price needed to meet the cost-effectiveness threshold to 2020 prices using the medical care component of the Consumer Price Index.

4.2 Updating drug prices

To determine whether drugs are currently priced at or below this cost-effectiveness threshold we will update estimated net prices by using data from SSR Health, LLC, the health care division of SSR, LLC, an independent investment research firm. To derive a net price, SSR Health combines data on unit sales with publicly disclosed US sales figures. Discounts, rebates, concessions to wholesalers and distributors, and patient assistance programs are subtracted from gross sales to derive a net price.

To estimate the most recent average net price in the US market, we will average net price data across the four most recently available quarters for which SSR data is available (October 2019-September 2020), to account for seasonal or other sources of annual price fluctuations. To confirm the validity of the SSR net prices, we will compare them to the Wholesale Acquisition Cost (WAC) and the Federal Supply Schedule Service (FSS). In cases where we deem the SSR net prices to be unreliable (such as the net prices being higher than the WAC), or where SSR prices are not available, we will use price estimates from FSS. If no data is available in either SSR or FSS, we will use list prices reported in Redbook. For physician administered drugs we will be using the same price data that was used in the report, which consists of the WAC price plus a markup.

SSR reports net prices on a per unit basis. We will convert the unit prices as listed in SSR to annual prices using the dosing assumptions used in the economic evaluation of our reports. For drugs with loading doses or dose-escalation regimens, we will use the maintenance dose to calculate annual costs (i.e., second year costs) for consistency. Drugs that require weight-based dosing will use the same weight assumptions as described in the economic evaluation section of our reports. The

remainder of partially used vials will be counted as medical waste. Pricing calculations and assumptions will be independently validated by another member of the research team and discrepancies will be resolved via a consensus process.

4.3 Final list

A final list of cost-effective drugs was generated using the methodology described above. Information on the cost-effective drugs will be abstracted according to the table shell below.

Table 4.1 Cost-Effective Drug List Table Shell

Drug Name Generic	Drug Name Brand	Indication	Route of Administration	Ceiling Price to Meet Cost-effectiveness Threshold	Current Price Estimate	Access and Affordability Alert?
Alemtuzumab	Lemtrada	Multiple Sclerosis	IV			
Dupilumab	Dupixent	Atopic dermatitis	SC			
Erenumab	Aimovig	Chronic Migraine	SC			
Fremanezumab	Ajovy	Chronic Migraine	SC			
Elagolix	Orilissa	Endometriosis	oral			
Onasemnogene Abeparvovec	Zolgensma	Spinal muscular atrophy	IV			
Tisagenlecleucel	Kymriah	Acute lymphoblastic leukemia	IV			
Infliximab	Remicade	Rheumatoid Arthritis	IV			
Infliximab	Remicade	Psoriasis	IV			
Guselkumab	Tremfya	Psoriasis	SC			
Apremilast	Otezla	Psoriasis	oral			
Brodalumab	Siliq	Psoriasis	SC			
Secukinumab	Cosentyx	Psoriasis	SC			
Ixekizumab	Taltz	Psoriasis	SC			
Ustekinumab	Stelara	Psoriasis	SC			
Afatinib	Gilotrif	EGFR Mutation-positive Metastatic Non-Small Cell Lung Cancer	oral			
Emicizumab	Hemlibra	Hemophilia A	SC			
Alirocumab	Praluent	Heterozygous familial	SC			

		hypercholesterolemia or CACVD				
Sacubitril/ Valsartan	Entresto	Congestive heart failure	oral			
Olaparib	Lynparza	Ovarian Cancer – Recurrent BRCA-Mutated	oral			
Axicabtagene ciloleucel	Yescarta	Adult aggressive B-Cell	IV			
Plasma-derived C1-INH	Haegarda	Hereditary Angioedema	SC			
Gefitinib	Iressa	Lung cancer – non small-cell (tkis)	oral			
Insulin Degludec	Tresiba	Diabetes	SC			
Ubrogepant	Ubrelvy	Acute Migraine	oral			
Rimegepant	Nurtec	Acute Migraine	oral			
Icosapent Ethyl	Vascepa	Cardiovascular Disease	oral			
Rivaroxoban	Xarelto	Cardiovascular Disease	oral			

5. List of Payers and Identification of Relevant Coverage Policies

We will review and abstract data from the largest formularies and coverage policies among 15 of the largest commercial payers (by covered lives) in the US as identified in the MMIT Analytics Market Access Database. Optum, one of the largest PBMs, is not included in the analysis because the details of its prior authorization policies were not available. The entity (payer or PBM) that controls the coverage decision is assigned the covered life. Medicare Private Drug Plans and Managed Care Plans and individual state Medicaid policies will not be evaluated in this review. The final list of payer formularies is listed in Table 5.1.

Table 5.1. Payer Formularies in Scope

Payer/PBM	Formulary
CVS Health (Aetna)	CVS Caremark Performance Standard Control w/ Advanced Specialty Control
Express Scripts PBM	Express Scripts National Preferred with Advantage Plus
UnitedHealth Group, Inc.	UnitedHealthCare Advantage Three Tier
CIGNA Health Plans, Inc.	Cigna Standard Three Tier
Kaiser Foundation Health Plans, Inc.	Kaiser Permanente Southern California
Anthem, Inc.	Anthem National Three Tier
MC-RX	MC-RX Formulary
Blue Cross Blue Shield of Massachusetts	BCBS Massachusetts Three Tier
Elixir PBM	Elixir Standard Formulary
Blue Shield of California	Blue Shield of California Plus Formulary
Health Care Service Corporation	BCBS of Illinois Basic 6 Tier
Florida Blue	Florida Blue Three Tier
Highmark, Inc.	Highmark Blue Cross Blue Shield 3 Tier
MedImpact Healthcare Systems, Inc.	MedImpact Portfolio High Formulary
Blue Cross Blue Shield of Minnesota	BCBS of Minnesota FlexRx Three Tier

6. Determination of Concordance of Coverage Policies with Fair Access Criteria

As mentioned earlier, the available coverage policies on cost-effective drugs will be evaluated to determine whether they meet a set of fair access criteria. Of course, there are many things that have to happen appropriately for patients to receive "fair access," and not all of these factors, including documentation burdens, and payer responsiveness to patients and clinicians, can be evaluated simply by reading written coverage policies. This project will therefore focus on several narrow elements that can be judged through available policies: cost sharing, clinical eligibility, restrictions on prescriber qualifications, and step therapy. For the cost-sharing criteria, "class" will be defined as drugs with the same mechanism of action or that are established as clinically equivalent options in clinical guidelines. The fair design criteria for these elements are describe in further detail below. All criteria are listed below, however not all will be evaluable at this stage of the project.

Table 6.1 Cost Sharing Fair Design Criteria

Cost Sharing	
Fair Access Criteria	In scope for this review?
Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price.	No
All medications identified by the IRS as high-value therapies should receive pre-deductible coverage within high deductible health plans.	No
At least one drug in every class should be covered at the lowest relevant cost-sharing level unless all drugs are priced higher than an established fair value threshold	Yes
If all drugs in a class are priced so that there is not a single drug that represents a fair value as determined through value assessment, it is reasonable for payers to have all drugs on a higher cost-sharing level.	Yes
If all drugs in a class are priced so that they represent a fair value, it remains reasonable for payers to use preferential formulary placement with tiered cost sharing to help achieve lower overall costs.	Yes
As part of economic step therapy, when patients try a lower cost option with a lower cost sharing level but do not achieve an adequate clinical response, cost sharing for further therapies should also be at the lower cost sharing level as long as those further therapies are priced fairly according to transparent criteria	No

See also Figure 6.1 for a visual representation of the cost sharing criteria algorithm.

Table 6.2 Clinical Eligibility Fair Design Criteria

Clinical Eligibility	
Fair Design Criteria	In scope for this review?
Payers should offer alternatives to prior authorization protocols such as programs that give feedback on prescribing patterns to clinicians or exempt them from prior authorization requirements ("gold carding") if they demonstrate high fidelity to evidence-based prescribing.	No
Payers should document at least once annually that clinical eligibility criteria are based on high quality, up-to date evidence, with input from clinicians with experience in the same or similar clinical specialty.	Yes
Clinical eligibility criteria should be developed with explicit mechanisms that require payer staff to document that they have: • Considered limitations of evidence due to systemic under-representation of minority populations; and • Sought input from clinical experts on whether there are distinctive benefits and harms of treatment that may arise for biological, cultural, or social reasons across different communities; and • Confirmed that clinical eligibility criteria have not gone beyond reasonable use of clinical trial inclusion/exclusion criteria to interpret or narrow the FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated.	Yes
For all drugs: Clinical eligibility criteria that complement the FDA label language may be used to: • Set standards for diagnosis; and/or • Define indeterminate clinical terms in the FDA label (e.g., "moderate-to-severe") with explicit reference to clinical guidelines or other standards; and/or • Triage patients by clinical acuity when the payer explicitly documents that triage is both reasonable and necessary because: ○ The size of the population included within the FDA label is extremely large, and there is a reasonable likelihood that many patients would seek treatment in the short term; AND ○ The clinical infrastructure is not adequate to treat all patients seeking care and/or broad coverage would create such substantial increases in short-term insurance premiums or other financial strain that patients would be harmed through loss of affordable insurance; AND	Yes

○ Acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm.	
For drugs with prices or price increases that have not been formally deemed unreasonable: Except for the three purposes outlined above, clinical eligibility criteria should not deviate from the FDA label language in a manner than would narrow coverage.	No
For drugs with prices or price increases that have not been formally deemed unreasonable: Documentation that patients meet clinical eligibility criteria should represent a light administrative burden, including acceptance of clinician attestation in lieu of more formal medical record documentation unless documentation is critical to ensure patient safety.	No
For drugs with prices or price increases that have been formally deemed unreasonable: Clinical eligibility criteria may narrow coverage by applying specific eligibility criteria from the pivotal trials used to generate evidence for FDA approval if implemented with reasonable flexibility and supported by robust appeals procedures as described in the implementation criteria.	No
For drugs with prices or price increases that have been formally deemed unreasonable: Documentation requirements to demonstrate that patients meet clinical eligibility criteria may represent a modest administrative burden, including requirements for medical record confirmation of key criteria instead of simple clinician attestation. In all cases, however, administrative burden should not result in major barriers to care for patients who meet criteria, and payers should perform and post publicly annual evaluations for each drug of rates of ultimate coverage approval following initial coverage denial due to documentation failures.	No

Table 6.3 Step Therapy Fair Design Criteria

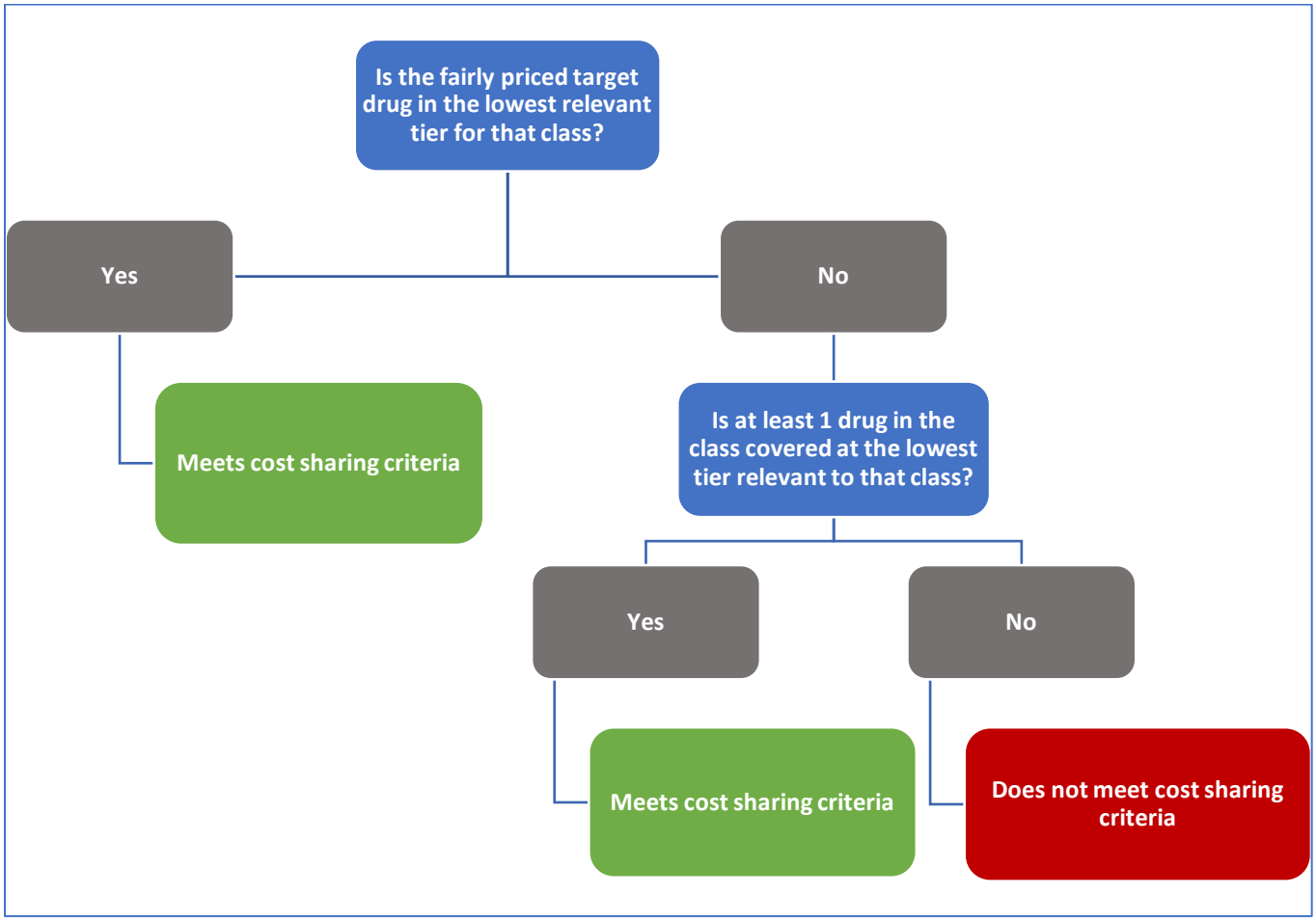
Step Therapy and Required Switching	
Fair Access Criteria	In scope for this review?
In order to justify economic step therapy policies as appropriate, payers should explicitly affirm or present evidence to document all of the following: • Use of the first-step therapy reduces overall health care spending, not just drug spending	No
• The first-step therapy is clinically appropriate for all or nearly all patients and does not pose a greater risk of any significant side effect or harm. • Patients will have a reasonable chance to meet their clinical goals with first-step therapy.	Yes

• Failure of the first-step drug and the resulting delay in beginning the second-step agent will not lead to long-term harm for patients. • Patients are not required to retry a first-line drug with which they have previously had adverse side effects or an inadequate response at a reasonable dose and duration.	
In order to justify required switching policies as appropriate, payers should explicitly affirm or present evidence to document all of the following: • Use of the required drug reduces overall health care spending.	No
• The required switch therapy is based on the same mechanism of action or presents a comparable risk and side effect profile to the index therapy. • The required switch therapy has the same route of administration or the difference in route of administration will create no significant negative impact on patients due to clinical or socio-economic factors. • Patients are not required to switch to a drug that they have used before at a reasonable dose and duration with inadequate response and/or significant side effects, including earlier use under a different payer.	Yes

Table 6.4 Provider Qualifications Fair Design Criteria

Provider Qualifications	
Fair Access Criteria	In scope for this review?
Restrictions of coverage to specialty prescribers are reasonable when payers explicitly affirm one or more of the following justifications: • Accurate diagnosis and prescription require specialist training, with the risk that non-specialist clinicians would prescribe the medication for patients who may suffer harm or be unlikely to benefit. • Determination of the risks and benefits of treatment for individual patients requires specialist training due to potential for serious side effects of therapy. • Dosing, monitoring for side effects, and overall care coordination require specialist training to ensure safe and effective use of the medication.	Yes
Requiring that non-specialist clinicians attest they are caring for the patient in consultation with a relevant specialist is a reasonable option when the condition is frequently treated in primary care settings but some elements of dosing, monitoring for side effects, and/or overall coordination of care would benefit from specialist input for many patients.	Yes

Figure 6.1 Cost-Sharing Fairness Criteria Algorithm



6.1 Process for comparing coverage policies to fair access criteria

For each drug, ICER research staff will summarize results of the policy abstraction data in Table 2 into a policy brief, which will also include details of the FDA label (including clinical trial eligibility criteria), clinical guidelines, and policy recommendations from ICER reports to provide relevant context. Research staff will make preliminary judgments regarding whether the coverage policy does or does not meet each fair design criterion, and then this judgment will be reviewed by an internist on the ICER staff. If the ICER clinician feels that clinical expert input is needed to determine whether a coverage policy meets the fair design criterion, ICER will seek to discuss the question with an expert involved in the original ICER report on that drug.

7. Analytic Plan

Our analyses will be both quantitative and qualitative in nature.

Quantitative analyses of the concordance of coverage policies with fair design criteria will examine:

Table 7.1. Rate of Concordance by Fair Design Criterion

Cost sharing	# of payer policies across all drugs meeting criteria/ all payer policies
Clinical eligibility criteria	# of payer policies across all drugs meeting criteria/ all payer policies
Step Therapy	# of payer policies across all drugs meeting criteria/all payer policies
Prescriber restrictions	# of payer policies across all drugs meeting criteria/all payer policies

Table 7.2. Rate of Concordance by Drug

	Cost Sharing	Clinical Eligibility Criteria	Step Therapy	Prescriber Restrictions
Drug 1	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies
Drug 2	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies	# of payer policies meeting criteria/ all payer policies

Table 7.3. Rate of Concordance by all Payers

Cost sharing	# of payers with >50% of policies across all drugs meeting criteria/# of payers
Clinical eligibility criteria	# of payers with >50% of policies across all drugs meeting criteria/# of payers
Step Therapy	# of payers with >50% of policies across all drugs meeting criteria/# of payers
Prescriber restrictions	# of payers with >50% of policies across all drugs meeting criteria/# of payers

Table 7.4. Rate of Concordance by Individual Payer

	Cost Sharing	Clinical Eligibility Criteria	Step Therapy	Prescriber Restrictions
Anthem (Largest Formulary)	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies
United Healthcare (Largest Formulary)	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies	# of policies across all drugs meet criteria/all policies

Additional quantitative analyses may be pursued to evaluate whether rates of concordance vary by route of administration, level of competition in the drug category, estimated eligible population, and other factors.

Qualitative information will be gathered from patient groups and clinical specialty societies to provide context to the quantitative analyses. The methods by which this information will be gathered is yet to be determined, but could include submission of published and unpublished data on barriers to access, examples of barriers to access that may reflect failure to meet fair access criteria or problems beyond those criteria evaluated directly in this report.

8. Payer and Patient Review Prior to Public Release

For any payer with policies judged not to meet fair access criteria ICER will provide them with the opportunity to review our judgment and provide comment if they feel the policy has been misinterpreted or misjudged. All payers will also be offered the opportunity to provide a written comment for inclusion with the material posted publicly when the report is released.

Draft results of the evaluation will also be shared with patient representatives of the Working Group to get feedback on how the fair access criteria are being judged across different coverage policies.

9. Changes in Process

Despite benefiting from the input of our Working Group, we expect that we will encounter situations throughout the research process that have not been fully anticipated. Thus, it should be expected that the fair access judging process and the analysis plan may change. ICER will be monitoring the process as it progresses and may need to alter aspects of the review if needed to maintain transparency and fairness to all parties. ICER commits to flexibility within this first review and to transparency about any needed changes.