Assessment of Barriers to Fair Access

Final Report

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Institute for Clinical and Economic Review

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List of Acronyms and Abbreviations Used in this Report

AAN	Allergy & Asthma Network
BCBS	Blue Cross Blue Shield
BSA	Body surface area
CA	California
COT	Continuation of Therapy
CSC	Cancer Support Community
evLYG	Equal value of life years gained
FDA	U.S. Food and Drug Administration
FL	Florida
FSS	Federal Supply Schedule
HCMA	Hypertrophic Cardiomyopathy Association
HCM	Hypertrophic Cardiomyopathy
HCSC	Health Care Service Corporation
HIX	Health Insurance Exchange
ICER	Institute for Clinical and Economic Review
IL	Illinois
MA	Massachusetts
MI	Michigan
NEA	National Eczema Association
NJ	New Jersey
OOP	Out-of-pocket
PBM	Pharmacy benefit manager
PhRMA	Pharmaceutical Research and Manufacturers of America
QALY	Quality-adjusted life year
QOL	Quality of life
US	United States
UT	Utah
VA	Virginia
VHA	Veterans Health Administration

Executive Summary

The national debate about drug pricing has largely focused on methods to determine whether the price of drugs are "fair" or "reasonable." A question far less examined is how to determine whether insurance coverage is providing fair access to drugs, whether they are fairly priced or not. To help address these questions, ICER worked with a broad set of stakeholders to develop a set of appropriateness criteria for pharmaceutical insurance coverage, as described in our 2020 white paper, Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals.

In this paper, we apply several key criteria from that white paper to the real-world coverage policies for 18 drugs reviewed by ICER in 2021: Benlysta and Lupkynis for lupus nephritis; Leqvio, Nexletol, and Nexlizet for high cholesterol; Abecma and Carvykti for multiple myeloma; Adbry, Cibinqo, Opzelura, and Rinvoq for atopic dermatitis; Camzyos for hypertrophic cardiomyopathy; Cinryze, Haegarda, and Tahkzyro for hereditary angioedema; Tezspire for asthma; Soliris and Vyvgart for myasthenia gravis. ICER has elected not to include aducanumab (Aduhelm™, Biogen) for the treatment of Alzheimer's in this assessment, as its supporting evidence base is so uncertain that determining whether fair access criteria should apply is not clear.

We assessed coverage policies for the selected drugs across 19 formularies, including the largest and smallest formularies by number of covered lives offered by the five largest commercial payers in the United States (US) the single formulary of the Veterans Health Administration (VHA), and the largest and smallest state health exchange plan formularies offered in the four geographic regions of the US (Northeast, Midwest, South, West). At the time we conducted our research, these formularies represented coverage policies governing pharmaceutical access for approximately 42 million Americans. We asked each payer to provide detailed coverage policy information to us, and if needed we supplemented payer submissions with information from the MMIT Analytics Market Access Database.

We rated the concordance of coverage policies against specific requirements of ICER's fair access criteria in four areas: 1) cost sharing to patients, with a single criterion requiring that fairly priced drugs or an equivalent option be placed on the lowest relevant tier of the formulary; 2) clinical eligibility, with criteria requiring that coverage for fairly priced drugs not be narrowed from the FDA label except to use clinical trial inclusion/exclusion criteria or clinical guidelines to define vague terms such as "moderate" or "severe;" 3) step therapy policies, requiring that each step meet standards for clinical appropriateness without a risk for irremediable harm to patients; and 4) provider qualification restrictions, where fair access requires that there be specific risk for misuse that merits restrictions to specialized prescribers. We also conducted exploratory analyses of whether, for each formulary, payers provide individuals shopping for health insurance sufficient

transparency into cost-sharing and tiering structure, clinical eligibility criteria, copay adjustment programs (e.g., copay accumulators and maximizers), and continuation of coverage policies. These exploratory analyses were conducted looking for information on the drugs Nexletol, Rinvoq, and Vyvgart.

Our analysis found that the rate of concordance for cost sharing was 62%, however it should be noted that this analysis applies only to the two drugs, Benlysta and Nexlizet, that were found to be fairly priced. Concordance was 99% for clinical eligibility criteria, 99% for step therapy, and 100% for provider restrictions. In part because of the very high concordance rates, we found no differences in concordance by size of formulary, whether in the private market or in state health exchange plans.

In the exploratory analyses of transparency of information for prospective plan members, all plans made cost-sharing and tiering information available to prospective members and clinical criteria for the three drugs selected were judged to be transparent between 70%-93% of formularies. Only 57% of formularies disclosed whether it participates in a copay adjustment program, and plans' presentation of information regarding potential continuation of coverage received the lowest scores, with adequate transparency in only 36% to 64% of all formularies.

Payers were given several weeks to provide comments on a draft of this assessment. As part of their feedback, one payer informed us that they had revised their coverage policy for Abecma in a way that would bring it into concordance with our fair access criteria.

There are several important limitations to these findings. First, we were unable to assess many important fair access criteria, including whether patient cost sharing is based on the plan's negotiated price for a drug rather than the drug's list price. Formulary tier placement is an imperfect analogue for cost sharing since specific cost sharing amounts and the choice of co-pay versus co-insurance are decided by the plan sponsor and not the payer. We were also unable to assess whether payers administered their policies (e.g., the process for requesting exceptions to medical coverage criteria) in line with our fair access criteria.

While the evidence available and the limitations of our research effort leave many questions, our results demonstrate that the majority of payer policies in the formularies evaluated are structured in a way to support many key elements of fair access. Concordance with our criteria for fair cost sharing was lowest at 62% across all formularies, but this analysis was limited by there being only two drugs whose net prices were found to meet cost-effectiveness standards, thereby making them eligible for fair access criteria evaluation. New to this year's assessment of fair access were our analyses of the transparency of coverage information that prospective members would find important in judging whether and how their current prescriptions would be covered. We found that many plans had inadequate information for prospective members, and even when it was available it was often placed on the provider portion of payer websites, raising the question of

whether prospective plan members would be able to find it. Similarly, information about the presence or absence of copay adjustment programs was often lacking. Health plans should take action to make this information more accessible to all prospective plan members as part of a general commitment to transparency and equity.

The final section of our assessment presents input received from patient organizations regarding barriers to fair access that their members are experiencing. Although it is anecdotal or relies on non-systematic surveys, this input supports findings from academic and professional society surveys demonstrating significant frustration with prior authorization and other features of insurance coverage, often to the point of creating significant delays in access to care. We acknowledge that our assessment cannot capture these procedures, and that even insurance coverage designed appropriately on paper can be administered with labyrinthine documentation procedures and other features that create important barriers to fair access.

This report therefore can have no simple conclusion on the degree of fair access to medications across the drugs and payers evaluated. Instead, we hope it will serve to foster further collaborative efforts to define the parameters of fair access and to work to elevate these ideals as a pillar of a just health care system.

1. Introduction

The national debate about drug pricing has focused 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 is 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 under which step therapy is a reasonable approach? When is it appropriate for the clinical criteria for coverage to be narrower than the Food and Drug Administration (FDA) labeled indication? And how should whether a drug is priced reasonably or not affect judgments of the appropriateness of certain strategies to manage its utilization?

To help address these questions, ICER has developed a set of appropriateness criteria for pharmaceutical insurance coverage, as described in our 2020 white paper, Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals. Readers of this current assessment are encouraged to read the earlier white paper to understand the broader ethical analysis and stakeholder input that were the foundation for these appropriateness criteria. This process featured a December 2019 ICER Policy Summit attended by representatives from patient groups, clinical specialty societies, private payers, and the life sciences industry.

The goal of this larger initiative from the outset has been for the "Fair Access" criteria to serve as a tool for assessment and as the starting point for dialogue and action to achieve fair access. In 2021, ICER applied a subset of the criteria to the coverage policies of leading commercial payers in our first Barriers to Fair Access Assessment. We produced another Fair Access Assessment in 2022 and intend to continue these reports on an annual basis. Based on our experience with our first reports, and with ongoing input from our multi-stakeholder Working Group, we have modified our methods for the 2023 report to expand the set of payer formularies evaluated within both the commercial and health exchange markets. We have also added analyses of new elements related to the transparency of coverage criteria for prospective enrollees.

These updates leave the basic approach largely consistent with that of the 2022 report. We asked each payer with formularies included in the scope to provide coverage policy information to us; we leveraged the MMIT Analytics Market Access Database to locate any policies not provided by payers. This year we are assessing coverage for the 18 drugs reviewed by ICER in 2021 and that are currently FDA approved for an indication consistent with the ICER review. Several drugs reviewed by ICER in 2021 were not approved by the FDA (roxadustat [AstraZeneca and FibroGen, Inc.] for

anemia in chronic kidney disease and baricitinib (Olumiant®, Eli Lilly, Incyte Corporation] for atopic dermatitis) or were withdrawn from the market (belantamab mafodotin [Blenrep™], GlaxoSmithKline for multiple myeloma) and were not included in this assessment. In addition, ICER has elected not to include aducanumab (Aduhelm™, Biogen) for the treatment of Alzheimer's in the assessment, as its supporting evidence base is so uncertain that determining whether fair access criteria should apply at all is not clear.

We assessed coverage policies for the selected drugs across 19 formularies, including the largest and smallest formularies by number of covered lives offered by the five largest commercial payers in the US, the single formulary of the Veterans Health Administration (VHA), and the largest and smallest state health exchange plan formularies offered in the four geographic regions of the US (Northeast, Midwest, South, West). At the time we conducted our research, these formularies represented coverage policies governing pharmaceutical access for approximately 42 million Americans. All payers and formularies except for VHA were identified using the MMIT Analytics Market Access Database.

As noted earlier, for the 2023 report, we have added two new transparency analyses. First, we have evaluated whether payers provide public access to policies regarding continuation of coverage (i.e., "grandfathering") for enrollees coming into an insurance plan. Second, we assessed whether payers make it clear in publicly available information whether their formularies are subject to copay adjustment programs (e.g., copay accumulators, maximizers). More detailed explanation of these methods changes is provided in the body of the report.

The key limitations of this analysis will be emphasized throughout the report and are summarized in Table 1 below. First, among the full set of fair access criteria contained in the white paper, many are not able to be assessed from an external review of insurance coverage and tiering information. The criteria that we cannot assess aim to lessen patient financial burden or represent standards for the internal process of using evidence to frame access restrictions, thus our inability to assess them reduces our ability to present a comprehensive judgment of whether payers are meeting fair access criteria. Second, for judgments on cost sharing, we could only use tiering as a signal of the relative magnitude of out-of-pocket payment required, an approach that does not capture the wide variety of levels of co-payments and co-insurance within any tiering structure that are selected not by the payer but by plan sponsors. And third, our focus within the commercial sector on the largest payers may skew our analysis toward formularies that are more, or less, in concordance with the fair access criteria than those of smaller payers around the country.

Table 1. Key Limitations to This Analysis of Barriers to Fair Access

Key Limitations
1. There are many important fair access design criteria not able to be evaluated from insurance coverage policies alone, including, for example: a. Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price; b. As part of 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 if those further therapies are priced fairly; c. Clinical eligibility criteria should be developed with explicit mechanisms that require payer staff to document that they have 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
2. We were unable to assess the efficiency of the process for requesting and adjudicating medical exceptions for individual patients.
3. Tiering as a surrogate for cost sharing is not able to reflect the actual out of pocket cost sharing amount nor whether co-payment versus co-insurance is required.
4. The data used to determine drug prices net of rebates are an average across all payers, including 340B institutions, and calculations include patient co-payment assistance and other expenditures that do not flow back to payers as rebates; therefore for any individual payer the net price they pay for a drug may not align precisely with our data, creating a risk for heterogeneity across payers in whether drugs have a "cost-effective" price and thus require preferential tiering to meet fair access criteria.
5. It is possible that the 10 commercial formularies selected for this assessment provide superior or inferior coverage than the formularies of smaller payers.

FDA: U.S. Food and Drug Administration

To help provide important guidance on this project, the Barriers to Fair Access Assessment has benefited from ongoing input from a multi-stakeholder Working Group consisting of several representatives from leading patient advocacy groups, two from clinical specialty societies, one from a pharmacy benefit manager, and one from an umbrella organization for life sciences companies. The Working Group has advised ICER on the application of the fair access criteria to coverage policies; provided insight into the patient experience with prescription drug coverage and access, including real-world examples; and advised on important nuances in the interpretation of payer coverage policies. None of them should be assumed to agree with any of the specific methods, findings, or perspectives presented in this report. Members of the Working Group are listed in the Supplemental Material.

2. Drugs and Formularies to be Assessed

The 18 drugs that are in scope for this year's report are shown in Table 2 below. Average net prices for these drugs between January 2022 and December 2022 were calculated based on data from SSR Health, LLC, an independent investment research firm. SSR Health estimates net price by calculating sales revenue net of all discounts, rebates, concessions to wholesalers and distributors, and the costs of patient assistance programs, and dividing this revenue by unit sales data. Table 2 below divides the list of drugs into those with net prices from SSR Health that fall above and below $150,000 per equal value of life years gained (evLYG), the threshold used for the purposes of this report to determine whether drugs are priced at a cost-effective level.

ICER did not calculate a benchmark price for two drugs in scope (Nexletol and Opzelura) so they were exempted from the cost sharing analysis. In the 2021 Atopic Dermatitis Report, Opzelura appeared to be more effective than medium potency topical corticosteroids in the mild-to-moderate population, but differences in trial designs precluded quantitative indirect comparisons across topical therapies. For Nexletol (bempedoic acid), ICER only estimated the cost effectiveness of the combination pill, Nexlizet (bempedoic acid/ezetimibe), as it is priced the same as Nexletol and so would be expected to dominate the monotherapy pill in any economic evaluation.

Table 2. Drug List

Brand Drug Name	Generic Drug Name	Condition	Maximum Annualized Cost-effective Price*
Drugs With Net Prices at or Below $150,000 per evLYG			
Benlysta®	Belimumab	Lupus Nephritis	$63,684
Nexlizet™	Bempedoic acid/ezetimibe	High Cholesterol	$2,705
Drugs With Net Prices Above $150,000 per evLYG Or Not Calculated			
Abecma®	Idecabtagene vicleucel	Multiple Myeloma	$275,734
Adbry®	Tralokinumab	Atopic Dermatitis	$36,418
Camzyos™	Mavacamten	Hypertrophic Cardiomyopathy	$15,608
Carvykti™	Ciltacabtagene autoleucel	Multiple Myeloma	$324,638
Cibinqo®	Abrocitinib	Atopic Dermatitis	$43,493
Cinryze®	C1 esterase inhibitors	Hereditary Angioedema	$146,243‡
Haegarda®	C1 esterase inhibitors	Hereditary Angioedema	$258,856‡
Leqvio®	Inclisiran	High Cholesterol	$6,243
Lupkynis™	Voclosporin	Lupus Nephritis	$104,988
Nexletol®	Bempedoic acid	High Cholesterol	Not calculated†
Opzelura™	Ruxolitinib	Atopic Dermatitis	Not calculated†
Rinvoq®	Upadacitinib	Atopic Dermatitis	$43,181
Soliris®	Eculizumab	Myasthenia Gravis	$20,186
Takhzyro™	Lanadelumab	Hereditary Angioedema	$228,749‡
Tezspire®	Tezepelumab	Asthma	$12,590
Vyvgart™	Efgartigimod	Myasthenia Gravis	$29,550

evLYG: Equal value of life years gained

*For details on dosing and pricing assumptions please see ICER Reports or ICER Analytics

† Indicates instances where ICER did not calculate a benchmark price. Since we did not calculate a benchmark price any item with this categorization will be exempt for the cost sharing analysis.

‡ The cost-effective price was determined by the QALY for drugs treating hereditary angioedema. The evLYG was not calculated in the ICER report assessing treatments for hereditary angioedema, as there was no evidence that these agents had any impact on mortality.

For these 18 drugs we conducted a standard data request from each payer to obtain cost sharing and prior authorization documentation for the relevant formularies. We used MMIT's Market Access Analytics platform to supplement any additional information that payers did not provide.

The 19 formularies evaluated are shown below in Table 3. Details on how MMIT assigns who "controls" a formulary and the covered lives under each formulary are provided in the Supplement. The formularies marketed under Express Scripts and Cigna Corporation remain separate in this database even though the companies are now merged because each company continues to make formulary decisions independently. Formularies for OptumRx and UnitedHealthcare were also evaluated separately because even though they use the same underlying template, UnitedHealthcare has the discretion to design its own coverage policies, which can differ from those in the OptumRx formulary.

Together, these formularies represent coverage policies governing pharmaceutical access for approximately 42 million Americans (MMIT Analytics as of 06/01/2023). See Table A4.1 in the Supplement for detailed information on covered lives per formulary.

Table 3. Largest and Smallest Formulary Offered by Each of the 5 Largest Commercial Payers, the VHA, and Largest and Smallest State Exchanges by Each Geographic Region per US Census

Payer	Formulary Name (Largest or Smallest)	Plan Type	Tiers Available
CVS Health (Aetna)	CVS Caremark Performance Standard Control w/Advanced Specialty Control (Largest)	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Generic or Non-Preferred Brand
CVS Health (Aetna)	CVS Aetna Standard Opt Out with Advanced Control Specialty Formulary (Smallest)	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Brand 4 - Preferred Specialty 5 - Non-preferred Specialty
Express Scripts PBM	Express Scripts National Preferred with Advantage Plus (Largest)	Commercial	1 - Formulary Generics 2 – Formulary brands 3 – Non-formulary brands
Express Scripts PBM	Express Scripts High Performance with Limited (Smallest)	Commercial	1 - Formulary generics 2 – Formulary brands
UnitedHealth Group, Inc.	UnitedHealthcare Advantage 3 Tier (Largest)	Commercial	1 – Lower-cost 2 – Mid-range cost 3 – Highest-cost
UnitedHealth Group, Inc.	UnitedHealthcare Flex Access 4 Tier (Smallest)	Commercial	1 – Lower-cost 2 – Mid-range cost 3 – Mid-range cost 4 – Highest-cost
OptumRx	OptumRx Select Standard Formulary (Largest)	Commercial	1 – Lower-cost 2 – Mid-range cost 3 – Higher-cost
OptumRx	OptumRx Premium Formulary (Smallest)	Commercial	1 – Lower-cost 2 – Mid-range cost 3 – Higher-cost

Payer	Formulary Name (Largest or Smallest)	Plan Type	Tiers Available
Cigna Corporation	Cigna Standard Three Tier (Largest)	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Generic or Non-Preferred Brand
Cigna Corporation	Cigna National Preferred (Smallest)	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Brand
Department of Veterans Affairs	VHA National Formulary	Federal	Not applicable
Horizon BlueCross Blue Shield of New Jersey	Horizon BlueCross Blue Shield of NJ HIX (Largest)	Health Exchange (NJ)	1 – Generic 2 – Preferred Brand 3 – Non-Preferred Brand
UnitedHealth Group, Inc.	UnitedHealthcare MA 3 Tier HIX (Smallest)	Health Exchange (MA)	1 – Lower-cost 2 – Mid-range cost 3 – Highest-cost
Health Care Service Corporation	Blue Cross Blue Shield of Illinois Marketplace 6 Tier HMO-HIX (Largest)	Health Exchange (IL)	1 – Generic 2 – Non-Preferred Generic 3 – Preferred Brand 4 – Non-Preferred Brand 5 – Preferred Specialty 6 – Non-Preferred Specialty
Quartz Health Solutions	Quartz Health Solutions Standard Choice Four Tier (Smallest)	Health Exchange (IL)	T1 – Generic T2 – Preferred Brand T3 – Non-Preferred Brand T4P – Preferred Specialty T4N – Non-Preferred Specialty
Florida Blue	Florida Blue Care Choices HIX (Largest)	Health Exchange (FL)	1 – Generic 2 – Preferred Brand 3 – Non-Preferred Brand 4 - Specialty
CVS Health (Aetna)	CVS Aetna Health Exchange Plan Innovation Health (Smallest)	Health Exchange (VA)	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Brand 4 - Preferred Specialty 5 - Non-preferred Specialty
Kaiser Foundation Health Plans, Inc.	Kaiser Permanente California HIX (Largest)	Health Exchange (CA)	1 - Generic 2 - Brand 4 - Specialty
Cambia Health Solutions	BridgeSpan Metallic Formulary HIX (Smallest)	Health Exchange (UT)	1 - Generic/Brand (high value) 2 - Generic/Brand (moderate value) 3 - Brand (moderate value) 4 - Brand (lower value) 5 - Specialty (moderate value) 6 - Specialty (low value)

CA: California, FL: Florida, HIX: Health Insurance Exchange, IL: Illinois, MA: Massachusetts, NJ: New Jersey, PBM: pharmacy benefit manager, UT: Utah, VA: Virgina, VHA: Veterans Health Administration

*Kaiser Permanente California HIX does not have a non-preferred brand tier (Tier 3).

3. Fair Access Criteria

There are many potential barriers to access spanning health literacy, disability status, provider education and availability, personal resources, and access to affordable insurance coverage. ICER's original white paper focused narrowly on two areas over which plan sponsors and payers (inclusive of both pharmacy benefit managers [PBMs] and insurers) have the most control: cost-sharing provisions and the design and implementation of utilization management.

Given this focus, the white paper did not address many other important areas of coverage policy, including thresholds for the number or type of drugs needed within drug classes; coverage for off-label prescribing; potential changes to the current rebate system; high-deductible benefit designs; and the role of co-payment coupons. Instead, the fair access criteria in the white paper directly address the following five domains:

- Cost-sharing provisions and tier placement as part of the drug benefit design
- Timing of development of prior authorization protocols following FDA approval
- Clinical eligibility criteria
- Step therapy and coverage requirements to switch medications
- Restrictions on prescriber qualifications

The purpose of the current assessment was to evaluate concordance of payer coverage policies for the 18 drugs that were the subject of ICER evidence reviews in 2021 and are currently FDA approved for the indication for which ICER evaluated them. In designing this assessment, we had to make one important concession: some of the fair access criteria cannot be evaluated without site visits, in-depth interviews, or access to material related to implementation of coverage policy procedures. Given our available resources, and the length of time it would take to perform a full, in-depth assessment of implementation, we decided not to evaluate the timing of development of prior authorization following FDA approval and several other important elements of fair access, such as responsiveness to initial requests for coverage, or timeliness of responses to requests for medical exceptions. We will stress throughout this report that these elements of fair access are critically important to patient and clinician experience and to patient outcomes. The fact that this current assessment did not evaluate these factors should be viewed as an important limitation on generalizing any judgment of whether a particular coverage policy represents "fair access."

In this year's report we have expanded our transparency analyses to include evaluation of whether plans provide adequate information to prospective enrollees on how continuation of coverage (i.e., "grandfathering") is managed and on whether formularies are subject to some form of adjustment to the way copayment support from drug makers is counted toward insurance deductible amounts (e.g. copay maximizers and accumulators). As with last year's transparency analyses, we chose to

perform a targeted evaluation of transparency limited to three drugs in the data set (Nexletol, Rinvoq, and Vyvgart). We selected these three drugs to ensure our analysis included treatments for different conditions and that medical and pharmacy benefit coverage would be assessed.

We present below and on the following pages the entire set of fair access criteria from the original white paper, indicating which criteria we have been able to include within the scope of this current assessment.

Table 4. Cost Sharing Fair Design Criteria

Cost Sharing	
Fair Design 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 Internal Revenue Service 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

Commentary on Assessment Strategy for Cost Sharing

We have evaluated cost-sharing concordance only on the basis of the tiering of a drug within the pharmacy benefit, even for those payers who provide coverage under both pharmacy and medical benefits. The rationale for this approach is that formulary tiers often do not exist within medical benefit designs. While we heard from payers that claims under the medical benefit may represent the vast majority of claims for certain drugs covered under both benefits, we felt it was important to evaluate cost sharing under the pharmacy benefit because it is more often under the control of the payer and should meet fair access criteria even if a very small number of patients are affected.

To meet the criterion for fair cost sharing, a fairly-priced drug or at least one of its equivalent options must be placed on the "lowest relevant" tier of the formulary. The interpretation of which is the lowest relevant tier for certain drugs is made difficult by the number and labeling of tiers in different formularies. For the purposes of this report, we required a fairly-priced drug to be placed in the second tier ("preferred brand") for formularies built with three or four tiers. Thus, even for

four-tier formularies with a single "specialty" fourth tier, the formulary was required to place these drugs on the second tier in order to be judged concordant.

This approach was informed by input from payers who noted that they ultimately have discretion on whether to place an expensive "specialty" drug on a lower tier. However, payers also noted that four-tier formularies are designed in conjunction with plan sponsors and that a payer has an implied responsibility to administer a four-tier formulary by putting all specialty drugs on the fourth tier. One payer also told us that many benefits consultants prefer to place all specialty drugs on the same tier because, in their view, this creates a "fair" system that is straightforward to administer and under which all individuals who need a specialty drug are subject to the same relative cost-sharing level. In addition, some plan sponsors may choose to have the same cost-sharing amount for drugs on a specialty fourth tier as on a preferred brand tier, eliminating the differential in out-of-pocket costs that would raise concerns for fair access. In fact, even though the principle of placing a drug on the lowest relevant tier should not change, the standard cost-sharing level for the specialty tier in some formularies may be lower than the cost-sharing level for the preferred brand tier in other formularies, complicating the attribution of a "barrier to fair access" based on tier placement alone.

The difficulty in interpreting tiering level as a surrogate for cost sharing is further compounded by the way tiered formularies are related to high-deductible health benefit designs. As shown in a report from Kaiser Family Foundation, in 2022, a higher percentage of all employees in plans without high deductibles had plans with four or more tiers (61%) than three tiers (30%). The 2021 version of the same report also noted that, whereas the percentage of four-tier formularies requiring co-insurance is higher than that for three-tier formularies (36% to 24%), most four-tier formularies still require only co-payments for all tiers.[1]

Thus, the correlation of tiering level and actual out-of-pocket cost is not exact across formularies. Our approach to evaluating tier placement emphasizes a judgment about relative cost sharing rather than absolute cost sharing, and the actual question of whether cost sharing is presenting an unfair barrier to access can only be answered at the level of the individual plan sponsor.

The existence of manufacturer coupons and other patient assistance programs further complicates the assessment of patient out-of-pocket costs. Manufacturer coupons and patient assistance programs defray some or all of the co-payment or co-insurance for a prescription and, when allowed, contribute toward deductibles and annual out of-pocket maximums. When no generic alternatives are available, these programs shield patients from the rising costs of branded drugs. However, while undoubtedly beneficial for individuals, these programs have been criticized for encouraging patients to take more expensive branded drugs when cheaper options are available, increasing plan spending and ultimately increasing the costs of pharmaceutical coverage.[2]

Benefit designs using either co-pay maximizer or accumulator mechanisms represent another important limitation in our ability to use formulary tiering as a surrogate for the cost sharing requirements for patients. Under co-pay accumulators, co-pay coupons and patient assistance programs are not applied to deductibles or out of pocket spending; patients must use the maximum amount of assistance for which they are eligible, after which they must meet their deductible and out-of-pocket spending obligations. The use of accumulators can result in increased payments from drugmakers to payers. This approach can subject patients to high out-of-pocket costs, sometimes even in cases where no cheaper generic alternatives are available. In contrast, maximizers set the patient's out of pocket expenses for the drug to the maximum amount of the assistance program and ensure those costs are spread over the full year. Maximizers typically shield patients from high out-of-pocket costs at the same time they increase manufacturer payments to insurers. Both of these programs, which are intended in part to address the aforementioned dynamic that incentivizes brand medications over less expensive alternatives, can serve more as a mechanism to reduce plan costs beyond what they would be even without the application of co-payment coupons or manufacturer assistance. Co-pay accumulators, in particular, can increase patients' cost burdens, especially in conditions for which there are no alternatives to branded medications.[3] The future of copay accumulators is uncertain, however, as a US judge recently struck down a rule that allowed them to be applied to brand drugs without generic equivalents.[4]

Despite these limitations in using tiering as an indicator of a fair approach to cost sharing, we believe that the general principle still holds: fairly-priced drugs should be placed on the lowest available relevant tier, which for brand name drugs is the second (preferred brand) tier.

Table 5. 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.	No
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.	No

Clinical Eligibility	
Fair Design Criteria	**In Scope for this Review?**
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 ○ Acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm.	Yes
For drugs with prices or price increases that have been deemed reasonable: Except for the three purposes outlined above, clinical eligibility criteria should not deviate from the FDA label language in a manner that would narrow coverage.	Yes
For drugs with prices or price increases that have been deemed reasonable: 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.	Yes
For drugs with prices or price increases that have been 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.	Yes

FDA: U.S. Food and Drug Administration

Table 6. Step Therapy and Required Switching Fair Design Criteria

Step Therapy and Required Switching	
Fair Design Criteria	**In Scope for this Review?**
In order to justify economic step therapy policies extending beyond FDA labeling 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. • 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.	Yes – threshold of a maximum of 3 steps even if all include appropriate first- line therapies
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. • 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.	No

FDA: U.S. Food and Drug Administration

Table 7. Provider Qualifications Fair Design Criteria

Provider Qualifications	
Fair Design Criteria	**In Scope for this Review?**
Restrictions of coverage to specialty prescribers are reasonable with 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

Table 8. Transparency Fair Design Criteria

Transparency	
Fair Access Criteria	**In Scope for this Review?**
Cost-sharing policies should be presented clearly to consumers prior to health plan selection, allowing all individuals to understand what cost sharing they will face for treatments they are currently taking or are considering.	Yes
Any significant change to formulary or cost sharing structures should not occur mid-cycle unless plan sponsors include this as a qualifying event allowing plan enrollees to switch plans.	No
At the point of care, clinicians and patients should be able to rapidly determine the cost-sharing requirements for any treatment along with cost sharing for other alternatives.	No
Individuals considering health plan enrollment should be presented with clear information allowing them to understand whether they meet the insurers' clinical criteria for the treatments they are currently taking. The policies should also set out the rationale behind them and be readily understandable.	Yes
Clinicians and patients should be able to rapidly determine the clinical criteria for any treatment and view the clinical rationale supporting these criteria. The referenced clinical information should be readily available to the prescribing/ordering provider and the public.	No
Individuals considering health plan enrollment should be presented with clear information allowing them to understand whether the treatments they currently take or envision taking will be subject to non-medical step therapy or switching policies.	Yes
Clinicians, pharmacists, and patients should be able to rapidly determine the requirements related to step therapy and switching policies and be able to easily view a full justification from the insurer.	No
Individuals considering health plan enrollment should be able to easily find information related to coverage criteria, including prescriber qualifications, for drugs that they or family members are currently taking.	Yes
Clinicians and patients should be able to rapidly determine whether there is a restriction on prescribing for any treatment. Insurers should provide ready assistance to primary care clinicians seeking connection with a relevant specialist for consultation as needed.	No

Commentary on Assessment Strategy for Transparency

To evaluate prospective plan enrollees' ability to find information about cost sharing (including whether a formulary is subject to a copay adjustment program), clinical eligibility criteria, and continuation of coverage policies for new plan members, we explored the public-facing website information for each formulary. The goal was to mimic the experience of a prospective enrollee who needed to find out what the requirements for coverage would be and the level of cost sharing for a drug they were already taking. We did not evaluate the transparency of this information during the process of care once patients are enrolled in the health plan.

4. Results

We evaluated coverage policies for 18 drugs across 19 formularies for a maximum of 18 x 19 = 342 possible drug-formulary policy combinations. During our data abstraction phase, the total possible drug-formulary policy combinations was reduced to 341 because the VHA has no national criteria for ruxolitinib cream (decisions about coverage and access are determined by local jurisdictions). In each category of fair access, some criteria were not applicable, either because the drug was not covered, the drug was not cost-effectively priced (in which case the cost-sharing fair access criterion related to tiering does not apply), or the drug was considered non-formulary (in which case only cost-sharing criteria can be assessed since payers can be held accountable for the tiering of therapeutic alternatives that are in the formulary).

Applicable policies on the 18 drugs were provided by all payers except for Quartz Health Solutions Standard Choice Four Tier. For this formulary we therefore used the MMIT database of coverage policies to inform our assessment, but full versions of policies for six drugs were not available. However, MMIT was able to provide some information abstracted from these policies, allowing us to evaluate step therapy on four of these drugs and prescriber criteria for one drug. All other fair access criteria for these six drugs were marked as "not applicable" for the Quartz Health Solutions Standard Choice Four Tier formulary. MMIT pulls data from a variety of sources known as the MMIT Network, a repository of open-source data including e-prescribing and similar point-of-care solutions, physician educational channels, long-term care and other pharmacies, pharmaceutical manufacturers, and most notably health plans and PBMs. When a policy is not referenced in the MMIT database, it is because MMIT has obtained this information either through a proprietary source, intelligence provided by their network of panelists, and/or other non-publishable digital data assets.

Throughout the report and supplement, numerators and denominators exclude policies for drugs that were determined to be non-formulary, except, as noted, for assessments of the cost-sharing criteria. While this approach does not explicitly penalize a payer in several categories of fair access for excluding a drug from the formulary, it avoids the concern that a payer could receive a favorable rating under clinical eligibility, prescriber restrictions, or step therapy even if the drug is substantially more difficult for a patient to access due to it being non-formulary.

Concordance by Fair Access Criterion

Our analysis of each individual drug-formulary combination is described in the Supplemental Material. As can be seen in Table 10 below, overall results for concordance with the four fair access criteria domains measured range from a low of 62% for cost sharing, to a high of 100% for prescriber restrictions.

Table 10. Number of Coverage Policies Available and Overall Rate of Concordance with Fair Access Criteria Assessed

Fair Access Criterion	Drug-Formulary Combinations with Relevant Policies Available* out of Applicable Policies, n/N (%)	Concordant Policies, n/N (%)
Cost sharing	37/38 (97%)	23/37 (62%)
Clinical eligibility	260/266 (98%)	257/260 (99%)
Step therapy	264/266 (99%)	261/264 (99%)
Prescriber restrictions	262/267 (98%)	262/262 (100%)

*No policies were provided by Quartz Health Solutions for the Quartz Health Solutions Standard Choice Four Tier. Full versions of policies for six drugs under the Quartz Health Solutions Standard Choice Four Tier were not available in MMIT. MMIT was able to provide some information abstracted from these policies, allowing us to evaluate step therapy on four of these drugs and prescriber criteria for one drug.

The percentage of policies judged concordant in Table 10 above uses the number of available policies as the denominator. We believe this is the best single quantitative measure of overall concordance because it does not seem reasonable to reduce concordance rates by including in the denominator policies that are not applicable. However, Table 11 below presents the results with not applicable drug policies as a component of all policies evaluated.

Table 11. Overall Rate of Concordance with Fair Access Criteria Assessed

Fair Access Criterion	Concordant n (%)	Not Concordant n (%)	Not Applicable* n (%)
Cost sharing	23 (7%)	14 (4%)	304 (89%)
Clinical eligibility	257 (75%)	3 (1%)	81 (24%)
Step therapy	261 (77%)	3 (1%)	77 (23%)
Prescriber restrictions	262 (77%)	0 (0%)	79 (23%)

*Not applicable includes cases when the drug is not covered by the payer, coverage status is unknown, and if a policy was not available (which was only the case for some drugs covered on the Quartz Health Solutions Standard Choice Four Tier). For cost sharing, the criteria are also not applicable for drugs that are not priced within cost-effectiveness levels or that are covered by a payer only through the medical benefit.

1. Cost Sharing

Two drugs out of the 18 were determined to be priced, net of rebates and discounts, within reasonable cost-effectiveness levels: Nexlizet and Benlysta.

Nexlizet had a 78% (14/18) concordance rate for cost-sharing, with three formularies (Cambia BridgeSpan Metallic Formulary HIX, CVS Aetna Health Exchange Plan Innovation Health, and Express Scripts High Performance) listing the drug as non-formulary, and one formulary (Horizon BlueCross BlueShield of NJ HIX) having the drug on formulary, but not having it placed on the lowest relevant tier.

Benlysta had a 47% (9/19) concordance rate for cost sharing, with ten formularies either listing the drug as non-formulary or placing it on a tier that did not represent the lowest relevant tier in that formulary. Five formularies list Benlysta on a specialty tier, but only one of these formularies had only one specialty tier available and was therefore rated as non-concordant (Florida Blue Care Choices HIX). Two health plans argued that placing Benlysta on the specialty tier was appropriate because a clinical guideline recommends other, less expensive treatments as first-line options.[5] However, we believe that steering patients to lower-cost options can be done more appropriately through step therapy because keeping a fairly priced drug on a tier requiring higher cost sharing is unfair to those patients who have tried other first-line options and not had adequate response.

- Non-formulary: CVS Aetna Health Exchange Plan Innovation Health, Kaiser Permanente California HIX
- Non-lowest relevant tier: CVS Aetna Standard Opt Out with ACSF, CVS Caremark Performance Standard Control w/Advanced Specialty Control, Cigna Standard Three Tier, OptumRx Premium, OptumRx Select Standard, HCSC Blue Cross Blue Shield of Illinois Marketplace 6 Tier HMO-HIX, Quartz Health Solutions Standard Choice Four Tier, Florida Blue Care Choices HIX

2. Clinical Eligibility

There was a high rate of concordance overall with the fair access criteria related to how payers used the FDA label, clinical trial clinical eligibility criteria, and clinical guidelines to determine the clinical eligibility criteria for coverage. Adbry was the only drug for which one or more formularies did not meet fair access criteria.

- Adbry: Three formularies (Cigna National Preferred, Express Scripts High Performance, and Express Scripts National Preferred) did not meet clinical eligibility criteria. These formularies define moderate to severe atopic dermatitis as only having an affected body surface area of greater than or equal to 10% with no mention of involved crucial body areas. Consensus recommendations state to consider involvement of crucial areas and quality of life in defining moderate to severe disease.

3. Step Therapy

There was a high rate of concordance for the design of step therapy policies. Soliris and Vyvgart were the only drugs for which one or more formularies did not meet fair access criteria.

- Soliris: Two formularies (Horizon BlueCross BlueShield of NJ HIX, and Cambia BridgeSpan Metallic Formulary HIX) required four steps of alternative therapies before accessing Soliris, exceeding the 3-step maximum to achieve concordance.

- Vyvgart: One formulary (Cambia BridgeSpan Metallic Formulary HIX) required four steps of alternative therapies before accessing Vyvgart, exceeding the 3-step maximum to achieve concordance.

As shown in Table 13 below, although no single drug had a rate of concordance for step therapy criteria lower than 89% across all formularies, we found variation for many drugs in the number of steps required before receiving coverage for the drug.

Table 13. Number of Steps Required for Prior Authorization by Drug

Drug Brand Name (Formulary type)	Most Common # of Steps	Range	Formularies with Non-Concordant Number of Steps (≥4 Steps)
Abecma (Medical)	0	0-1	N/A
Adbry (Pharmacy)	0	2	N/A
Benlysta (Pharmacy)	0	0	N/A
Camzyos (Pharmacy)	0	0-2	N/A
Carvykti (Medical)	0	0-1	N/A
Cibinqo (Pharmacy)	0 or 1	0-2	N/A
Cinryze (Pharmacy)	0	0-1	N/A
Haegarda (Pharmacy)	0	0-1	N/A
Leqvio (Medical)	1	0-3	N/A
Lupkynis (Pharmacy)	0	0	N/A
Nexletol (Pharmacy)	1	0-1	N/A
Nexlizet (Pharmacy)	1	0-1	N/A
Opzelura (Pharmacy)	0	0	N/A
Rinvoq (Pharmacy)	0	0-2	N/A
Soliris (Medical)	3	0-4	Cambia BridgeSpan Metallic Formulary HIX, Horizon BlueCross BlueShield of NJ HIX
Takhzyro (Pharmacy)	0	0-1	N/A
Tezspire (Pharmacy)	0	0-1	N/A
Vyvgart (Medical)	2	0-4	Cambia BridgeSpan Metallic Formulary HIX

HIX: Health Insurance Exchange, N/A: Not Applicable, NJ: New Jersey

4. Prescriber Restrictions

Of the applicable policies, all 262/262 (100%) were concordant with the fair access criteria for prescriber restrictions.

Concordance by Drug

Because the drugs included in our analysis can be covered under pharmacy benefits, medical benefits, or both, we had to decide how to report the findings in a way that conveys a fair "apples to apples" comparison across formularies. When a drug was covered by a payer under both the pharmacy benefit and medical benefit, we selected for assessment the coverage policy under the benefit type that was used by the greatest number of payers overall (i.e., the "predominant benefit plan type"). Only pharmacy benefit coverage policies were used to judge cost-sharing concordance for reasons discussed above in Chapter 3. Results for each drug on concordance on all criteria are shown on the following page in Table 14.

Because overall concordance with the fair access criteria was so high, there was not enough variation to explore correlation with features of the drug, drug class, or drug pricing.

Table 14. Concordance with Fair Access Criteria by Drug: Number (%) of Payers with Concordant Policies out of Payers with Applicable Policies. Concordance Requires Meeting All Applicable Individual Criteria.

	Predominant Benefit Plan Type	**Cost Sharing**	**Clinical Eligibility**	**Step Therapy**	**Prescriber Restrictions**
Drug (Indication)	*(Number of formularies with predominant plan type/number of all formularies)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
Abecma (Multiple Myeloma)	Medical (19/19)	N/A	13/13 (100)	14/14 (100)	14/14 (100)
Adbry (Atopic Dermatitis)	Pharmacy (19/19)	N/A	13/16 (81)	16/16 (100)	16/16 (100)
Benlysta (Lupus Nephritis)	Pharmacy (19/19)	9/19 (47)	17/17 (100)	17/17 (100)	17/17 (100)
Camzyos (Hypertrophic Cardiomyopathy)	Pharmacy (19/19)	N/A	14/14 (100)	14/14 (100)	15/15 (100)
Carvykti (Multiple Myeloma)	Medical (18/18)	N/A	13/13 (100)	13/13 (100)	13/13 (100)
Cibinqo (Atopic Dermatitis)	Pharmacy (19/19)	N/A	14/14 (100)	14/14 (100)	14/14 (100)
Cinryze (Hereditary Angioedema)	Pharmacy (14/19)	N/A	10/10 (100)	11/11 (100)	10/10 (100)
Haegarda (Hereditary Angioedema)	Pharmacy (18/19)	N/A	16/16 (100)	17/17 (100)	16/16 (100)
Leqvio (High Cholesterol)	Medical (15/19)	N/A	15/15 (100)	15/15 (100)	15/15 (100)
Lupkynis (Lupus Nephritis)	Pharmacy (19/19)	N/A	12/12 (100)	12/12 (100)	12/12 (100)
Nexletol (High Cholesterol)	Pharmacy (19/19)	N/A	13/13 (100)	13/13 (100)	13/13 (100)
Nexlizet (High Cholesterol)	Pharmacy (19/19)	14/18 (78)	13/13 (100)	13/13 (100)	13/13 (100)
Opzelura (Atopic Dermatitis)	Pharmacy (18/18[†])	N/A	10/10 (100)	10/10 (100)	10/10 (100)
Rinvoq (Atopic Dermatitis)	Pharmacy (19/19)	N/A	19/19 (100)	19/19 (100)	19/19 (100)
Soliris (Myasthenia Gravis)	Medical (13/19)	N/A	18/18 (100)	16/18 (89)	18/18 (100)
Takhzyro (Hereditary Angioedema)	Pharmacy (18/19)	N/A	17/17 (100)	18/18 (100)	17/17 (100)
Tezspire (Asthma)	Pharmacy (13/19)	N/A	14/14 (100)	14/14 (100)	14/14 (100)
Vyvgart (Myasthenia Gravis)	Medical (15/19)	N/A	16/16 (100)	15/16 (94)	16/16 (100)

N/A: Not applicable (meaning that these drugs are not priced at a cost-effective level (n=2) and therefore the cost sharing criteria do not apply)
*The total N for each fair access criteria represents whether the specific criterion is applicable for that drug.
† The number of formularies is 18 for Opzelura (ruxolitinib cream) because the VHA National Formulary has no national criteria for this agent (decisions about coverage and access are determined by local jurisdictions)

Concordance by Formulary

As shown in Table 15 on the following page, the percent concordance across all 18 drugs on specific fair access criteria varies between formularies, ranging from 0% to 100% for cost sharing, 90% to 100% for clinical eligibility, and 75% to 100% for step therapy. One limitation in interpreting the specific findings for individual formularies should be emphasized: the relatively small number of drug policies applicable for assessment, particularly in the cost sharing domain. The small number of relevant policies in this domain, ranging from one to two, means that a different rating for a single drug leads to very large absolute differences in the percentage of concordance with fair access criteria. Therefore, we advise readers of these results to avoid making strong interpretations of relative performance as measured in percentage for concordance with the cost sharing criteria.

It should also be noted that not all formularies could be assessed on all criteria for the full set of 18 drugs. In Table 15, for each formulary, the total 'N' between criteria differs across payers when some payers covered particular drugs only on the medical benefit, or when drugs were excluded from the formulary. As mentioned, the cost-sharing criteria are only applicable if the drug is priced at a cost-effective level and is covered by the payer under a pharmacy benefit. For non-formulary drugs, cost-sharing criteria are applicable since they can be applied to the formulary placement of reasonable alternatives in the same drug class, but the remaining criteria would not apply.

Table 15. Rate of Concordance by Formulary: Number (%) of Policies Meeting Each Fair Access Criterion out of all Relevant Policies

		Cost Sharing*	Clinical Eligibility	Step Therapy	Prescriber Restrictions
Formulary	Formulary Size and Region	*Concordant Policies, n/N† (%)*			
Commercial					
Cigna National Preferred	Small	2/2 (100)	17/18 (94)	18/18 (100)	18/18 (100)
Cigna Standard Three Tier	Large	1/2 (50)	16/16 (100)	16/16 (100)	16/16 (100)
CVS Aetna Standard Opt Out with ACSF	Small	1/2 (50)	14/14 (100)	14/14 (100)	14/14 (100)
CVS Caremark Performance Standard Control w/Advanced Specialty Control	Large	1/2 (50)	9/9 (100)	9/9 (100)	9/9 (100)
Express Scripts High Performance	Small	1/2 (50)	9/10 (90)	10/10 (100)	10/10 (100)
Express Scripts National Preferred	Large	2/2 (100)	10/11 (91)	11/11 (100)	11/11 (100)
OptumRx Premium	Small	1/2 (50)	11/11 (100)	11/11 (100)	11/11 (100)
OptumRx Select Standard	Large	1/2 (50)	16/16 (100)	16/16 (100)	16/16 (100)
UnitedHealthcare Flex Access 4 Tier	Small	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)
UnitedHealthcare Advantage 3 Tier	Large	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)
Federal					
VHA National Formulary	N/A	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)
State Exchange					
Cambia BridgeSpan Metallic Formulary HIX	Small, West	1/2 (50)	8/8 (100)	6/8 (75)	8/8 (100)
CVS Aetna Health Exchange Plan Innovation Health	Small, South	0/2 (0)	6/6 (100)	6/6 (100)	6/6 (100)
Florida Blue Care Choices HIX	Large, South	1/2 (50)	18/18 (100)	18/18 (100)	18/18 (100)
HCSC Blue Cross Blue Shield of Illinois Marketplace 6 Tier HMO-HIX	Large, Midwest	1/2 (50)	15/15 (100)	15/15 (100)	15/15 (100)
Horizon BlueCross BlueShield of NJ HIX	Large, Northeast	1/2 (50)	14/14 (100)	13/14 (93)	15/15 (100)
Kaiser Permanente California HIX	Large, West	1/2 (50)	18/18 (100)	18/18 (100)	18/18 (100)
Quartz Health Solutions Standard Choice Four Tier‡	Small, Midwest	0/1 (0)	8/8 (100)	12/12 (100)	9/9 (100)
UnitedHealthcare MA 3 Tier HIX	Small, Northeast	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)

BCBS: Blue Cross Blue Shield, CA: California, HCSC: Health Care Service Corporation, HIX: Health Insurance Exchange, MA: Massachusetts, MI: Michigan, NJ: New Jersey, PBM: Pharmacy Benefit Manager, VHA: Veterans Health Administration

*Five formularies list Benlysta on a specialty tier; only one of these formularies had a single one specialty tier available and was therefore rated as non-concordant (Florida Blue Care Choices HIX).

†Cost-sharing criteria apply to only two drugs that were priced at cost-effective levels. N for the remaining three criteria may not always total 18 due to criteria being not applicable for some drugs.

‡Full versions of policies for six drugs under Quartz Health Solutions Standard Choice Four Tier were not available in MMIT. MMIT was able to provide some information abstracted from these policies, allowing us to evaluate step therapy on four of these drugs and prescriber criteria for one drug.

Concordance by Formulary Type, Size and Location

Tables 16 through 19 report the rate of concordance by formulary type, size, and location.

There were high rates of concordance for clinical eligibility, step therapy and prescriber restrictions regardless of formulary type, size, and location (≥92%). Given the high concordance rates through all categories of formularies, there was no signal of systematically better or worse performance by formulary type, size, or location. Because of the small number of relevant policies in the cost sharing domain, we advise readers to avoid drawing strong conclusions of relative performance by formulary type, size, or location for this domain.

Table 16. Rate of Concordance by Formulary Type

	Cost Sharing	**Clinical Eligibility**	**Step Therapy**	**Prescriber Restrictions**
Formulary type	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
State exchange formularies	7/15 (47)	104/104 (100)	105/108 (97)	106/106 (100)
Commercial formularies	14/20 (70)	136/139 (98)	139/139 (100)	139/139 (100)
Federal (VHA National) formulary	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)

*The total N for each fair access criteria represents whether the specific criterion is applicable for the drug and formulary combination within each formulary type.

Table 17. Rate of Concordance by Formulary Size

	Cost Sharing	**Clinical Eligibility**	**Step Therapy**	**Prescriber Restrictions**
Formulary Size	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
Small	10/17 (59)	107/109 (98)	111/113 (98)	110/110 (100)
Large	11/18 (61)	133/134 (99)	133/134 (99)	135/135 (100)
VHA National formulary	2/2 (100)	17/17 (100)	17/17 (100)	17/17 (100)

*The total N for each fair access criteria represents whether the specific criterion is applicable for the drug and formulary combination within each formulary size.

Table 18. Rate of Concordance by Health Exchange Formulary Size

	Cost Sharing	**Clinical Eligibility**	**Step Therapy**	**Prescriber Restrictions**
State Exchange Formulary Size	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
Small	3/7 (43)	39/39 (100)	41/43 (95)	40/40 (100)
Large	4/8 (50)	65/65 (100)	64/65 (98)	66/66 (100)

*The total N for each fair access criteria represents whether the specific criterion is applicable for the drug and formulary combination within each health exchange formulary size.

Table 19. Rate of Concordance by Health Exchange Location

	Cost Sharing	Clinical Eligibility	Step Therapy	Prescriber Restrictions
State Exchange Location	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
Midwest	1/3 (33)	23/23 (100)	27/27 (100)	24/24 (100)
Northeast	3/4 (75)	31/31 (100)	30/31 (97)	32/32 (100)
South	1/4 (25)	24/24 (100)	24/24 (100)	24/24 (100)
West	2/4 (50)	26/26 (100)	24/26 (92)	26/26 (100)

*The total N for each fair access criteria represents whether the specific criterion is applicable for the drug and formulary combination within each state exchange location.

Concordance by Condition

Table 20 below reports the rate of concordance by condition.

There was 100% concordance for drugs included in our analysis for treating hereditary angioedema, multiple myeloma, hypertrophic cardiomyopathy, and asthma. For atopic dermatitis, there were three formulary policies for Adbry that had clinical eligibility restrictions that did not meet our concordance criteria. For lupus nephritis, the majority (53%) of formulary policies for Benlysta had cost sharing criteria that did not meet concordance. For high cholesterol, four formulary policies for Nexlizet had tiering that did not meet our cost sharing criteria. For myasthenia gravis, there were three formulary policies (two for Soliris and one for Vyvgart) that had step therapy restrictions that did not meet our concordance criteria.

Table 20. Rate of Concordance by Condition

	Cost Sharing	Clinical Eligibility	Step Therapy	Prescriber Restrictions
Condition	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*	*Concordant Policies, n/N* (%)*
Atopic Dermatitis	0/0 (100)	56/59 (95)	59/59 (100)	59/59 (100)
Lupus Nephritis	9/19 (47)	29/29 (100)	29/29 (100)	29/29 (100)
High Cholesterol	14/18 (78)	41/41 (100)	41/41 (100)	41/41 (100)
Hereditary Angioedema	0/0 (100)	43/43 (100)	46/46 (100)	43/43 (100)
Multiple Myeloma	0/0 (100)	26/26 (100)	27/27 (100)	27/27 (100)
Myasthenia Gravis	0/0 (100)	34/34 (100)	31/34 (91)	34/34 (100)
Hypertrophic Cardiomyopathy	0/0 (100)	14/14 (100)	14/14 (100)	15/15 (100)
Asthma	0/0 (100)	14/14 (100)	14/14 (100)	14/14 (100)

*The total N for each fair access criteria represents whether the specific criterion is applicable for the drug and formulary combination within each condition.

Exploratory Transparency Analyses

In addition to analyses of concordance with fair access criteria for cost sharing, clinical eligibility criteria, step therapy, and prescriber restrictions, we evaluated a select set of drugs and formularies on criteria related to the transparency of information for prospective enrollees on cost sharing (including whether a formulary is subject to a copay adjustment program [e.g., accumulators or maximizers]), clinical eligibility criteria, and continuation of coverage when switching from an outside insurer.

We used a targeted approach and elected to evaluate the availability of this information for three drugs: Nexletol, Rinvoq, and Vyvgart. We excluded formularies offered by PBMs from all transparency analyses because their typical business model is to provide services to a health plan, which then serves as the primary portal through which potential enrollees learn about coverage for drugs that they are already taking or expecting to begin. In addition, health plans frequently request changes to the standard policies offered by PBMs; as such, the documents available from a payer website are the most accurate representation of the policies that apply to plan members. After excluding PBMs, 14 formularies were assessed for transparency.

For each of these analyses, we conducted a search of the payer's website to determine whether this information was available to individuals prior to plan enrollment. We reviewed information posted under the patient portal, provider portal, and used the search function of the website when policies could not otherwise be located. We note that our use of the provider portal is a liberal interpretation of making this information transparent for prospective enrollees; however, because information through the provider portal is technically available publicly to individuals prior to plan enrollment, we considered documents found on the provider portal to be eligible for consideration. We did not use external search engines (e.g., Google) to supplement this search because there would not have been a definitive and consistent way to determine whether a policy identified through this method was current and/or whether it applied to one of the formularies in scope.

In keeping with our approach throughout this report, non-formulary drugs were not assessed for transparency of clinical criteria and received a "not applicable" (N/A) rating. Similarly, we did not assess the transparency of cost sharing information for drugs covered under the medical benefit, as formulary tiers often do not exist within medical benefit designs. This resulted in denominators of less than 14 for several of the below analyses.

Summary results are presented in Table 21 below and ratings for each formulary / drug combination can be found in Supplement Tables B20.1-B20.3.

Table 21. Summary of Results for Exploratory Transparency Analyses

	Transparency of Cost-Sharing and Tier Information	Transparency of Copay Adjustment Programs	Transparency of Clinical Criteria	Transparency of Continuation of Coverage
Nexletol	10/10 (100%)	8/14 (57%)	7/10 (70%)	5/14 (36%)
Rinvoq	13/13 (100%)	8/14 (57%)	12/13 (92%)	9/14 (64%)
Vyvgart	3/3 (100%)	8/14 (57%)	13/14 (93%)	9/14 (64%)

Transparency of Cost Sharing and Copay Adjustment Programs

For cost sharing, a plan met criteria if they provided information showing the tiers for their formulary and the specific cost-sharing or co-insurance levels for each tier. For copay adjustment programs, a plan met criteria if they disclosed that patients might be subject to one of these programs and provided information about whether it applies to some or all drugs.

All formularies that covered the assessed drugs provided sufficient information on cost-sharing under the pharmacy benefit (10/10 for Nexletol, 13/13 for Rinvoq, and 3/3 for Vyvgart; lower denominators are due to non-formulary status for Rinvoq and Nexletol, and medical benefit coverage for Vyvgart).

Fewer formularies (8 [57%] of 14 for each drug) provided information on whether the drugs were subject to copay adjustment programs. Two plans (Kaiser Permanente and United Healthcare) provided this information in a particularly clear manner. Kaiser Permanente has a dedicated webpage that lists specific drugs that are eligible for coupons, explains when patients can use them, where they can be found, and specifies that coupons count toward out-of-pocket obligations.[6] United Healthcare has a document that notes that specialty medications filled at the Optum Pharmacy are subject to a copay accumulator program and describes how patients' out-of-pocket obligations have changed since the implementation of this program.[7]

Transparency of Clinical Criteria

When a copy of the clinical policy for Nexletol, Rinvoq, or Vyvgart was publicly accessible on the plan's website, meaning no login credentials were required, the plan was considered to have transparency of clinical criteria. The majority of formularies 12 (92%) of 13 for Rinvoq (the lower denominator is due to non-formulary status) and 13 (93%) of 14 for Vyvgart provided public access to the clinical criteria. A slightly lower percentage was found for Nexletol (7 [70%] of 10). An important limitation of these findings is that more than half of the clinical criteria policies across the three drugs were only found within the provider portal sections of the plans' websites. It is possible that a prospective plan member would not explore the Provider section of a website for materials, especially in the cases where a plan requires the user to attest that they are a health care provider. Cambia is an example of a plan that provides access to their clinical criteria for all three drugs through the member section of their website.[8]

The plan offered by Kaiser Permanente presents an unusual circumstance. Although the formulary did not meet our criteria for transparency (no detailed policies were publicly available), we understand that physicians working in the Kaiser Permanente system are able to prescribe any drug they deem to be medically necessary, even if it is not a recommended therapy under their internal guidelines. Had Kaiser Permanente described this practice on their website, they would have met our criteria.

Transparency of Continuation of Coverage Policies

We assessed whether plans provide explicit policies that describe the process through which prospective plan members can request continued coverage for their drug when switching insurers to join the plan. As there may be alternative terms for this process used across the health care space, we searched for a broad set of terms within each plan's website, such as "grandfathering," "exception request," "continuation of therapy/coverage," and "maintenance of medical coverage."

We found that only five (36%) of 14 formularies had adequate transparency on clinical criteria for Nexletol while nine (64%) of 14 formularies had clinical criteria available for Rinvoq and Vyvgart. In some cases, there was inconsistent transparency within a given formulary. For example, the policy for Vyvgart in the Florida Blue Care Choices HIX formulary clearly states that, "an authorization or reauthorization for efgartigimod [Vyvgart] has been previously approved by Florida Blue or another health plan…"[9] In contrast, the same policy for Nexletol from the same formulary says only that, "the patient has been previously approved for the requested agent through the plan's Prior Authorization process," which does not specifically describe whether this policy applies to new members who are already taking the drug and, as such, did not meet our transparency criterion.[10]

In general, there was substantial ambiguity in the language that many plans used to describe their policies for continuation of coverage. We found that plans use different terms to describe their continuation of coverage policies for incoming new plan members, such as "exception request," "continuation of therapy/coverage," and "manual transition of care." One plan (HCSC) told us the absence of any language regarding continuation of coverage for Nexletol meant the member would need to meet HCSC's initial authorization criteria. However, because the plan did not provide explicit language stating new plan members need to go through HCSC's criteria, we found the policy to be insufficiently transparent. In contrast, Cambia has a standalone "continuation of therapy (COT)" policy which clearly notes that a new plan member must meet Cambia's medical necessity criteria to receive coverage for a non-formulary drug.[11]

Furthermore, some formularies had policies that described how continuation of coverage would be managed for non-formulary drugs, but not for drugs included on the formulary that had prior authorization or step therapy criteria. For example, CVS Aetna Standard Opt Out with ACSF formulary did not describe how continuation of coverage would be managed for Nexletol, which is on the preferred brand tier.[12] In contrast, Nexletol is non-formulary for CVS Aetna Health Exchange

Plan Innovation Health, but the plan transparently describes how patients can request a medical exception to allow coverage for a drug they are currently taking.[13] This discrepancy may be due to rules governing the transparency of benefit, coverage and cost-sharing information required by CMS of health insurance exchange plans.[14]

Changes to Payer Coverage Policies After Draft Analysis

Draft results of this analysis were shared with all payers on June 1, 2023 for a three-week comment period, and again on August 29 for a four-week comment period. During each period, payers were asked to submit comments and were invited to provide corrections, updates, and perspectives on the draft concordance ratings. As part of the feedback received from payers, one payer informed us that they were changing their clinical criteria for coverage in a way that would now meet our fair access criteria. In order to preserve the integrity of the analysis, we have not included this change in the primary results presented above. But to capture the status of all policies as of the time of the publication of this report, and to suggest how coverage policies may evolve to meet fair access criteria, we summarize this change in Table 22 below and present an updated overall concordance rating on clinical criteria for the affected formulary.

Table 22. Changes to Payer Policies After June 1, 2023 That Achieved Concordance with Fair Access Criteria

Formulary	Drug	Policy Change	Concordance with Policy Change Included
Cigna National Preferred	Adbry	Effective October 1, 2023, the Cigna National Preferred formulary no longer defines moderate to severe atopic dermatitis as having an affected body surface area of ≥10%, regardless of involvement of crucial body areas.	Clinical Criteria 18/18 (100%)

5. Input from Patient Groups

To enhance our understanding of real-world patient experience with access to prescription medications, we reached out to 12 disease-specific patient organizations across the eight therapeutic areas represented by the drugs in this year's report. The following groups submitted comments and evidence to describe their community's experience with access challenges:

- Allergy & Asthma Network (AAN)
- Cancer Support Community (CSC)
- Hypertrophic Cardiomyopathy Association (HCMA)
- National Eczema Association (NEA)

The above groups have documented through surveys and patient stories the substantial financial and health impacts of not having appropriate and timely access to care. These challenges are described below to serve as a reminder of the realities faced by people across our health system, but it is important to note that these examples are not necessarily the result of any payer policies evaluated in this report.

From the asthma community, we heard that out-of-pocket (OOP) costs in the form of co-insurance or co-payments can be as high as $3,000 per month (as a reference, the Affordable Care Act maximum OOP is $9,100 for an individual and $18,200 for a family).[15] This forces some people to choose between paying for their medication or for housing and food, or making car payments, suggesting that this type of cost-sharing is not equitable for the larger patient community. In addition, although the following drugs were not in scope for this year's report, these patient quotes highlight current coverage and cost-sharing challenges with asthma medications:

> "Insurance carrier wanted the insurance member (me) to pay for biologic. Could not afford the price." (Xolair)
>
> "I have only been able to obtain samples from my provider. The insurance has denied multiple appeals for me to receive this medication. I have stopped taking it because I cannot pay for it without insurance." (Dupixent)
>
> "It is not covered under my insurance. Nothing similar either is covered. I'm currently using Dupixent in a study and love it but won't be able to use it after August." (Dupixent)

A 2020 analysis of multiple myeloma patients in the U.S. who participated in Cancer Support Community's (CSC's) Cancer Experience Registry showed that nearly half reported they were moderately to very seriously concerned about health insurance or money worries. Four out of 10 patients reported depleting savings or using money from retirement to cover myeloma treatment

costs. One out of four patients spent over $500 in monthly out-of-pocket costs to cover cancer care.[16]

In a separate CSC analysis of 313 multiple myeloma and 122 chronic lymphocytic leukemia patients, 29% reported depleting their savings, 19% reported borrowing against or using money from a retirement plan, 13% reported liquidating assets, 7% reported collecting unemployment insurance, 4% reported taking an extra job, 4% reported choosing a less effective treatment, 4% reported cashing in a life insurance policy early, and 2% reported having their house foreclosed due to the financial costs of cancer care. Based on the percent reporting "sometimes," "often," or "always" engaging in behaviors related to postponing care, 12% postponed doctor's appointments, 5% postponed follow-up screening or blood work, 6% postponed filling prescriptions, 5% skipped dosages of prescribed drugs, 12% delayed complementary treatment including therapy, and 16% postponed psychological counseling or support.

Age, lower income, OOP costs, time since diagnosis, and advanced stage all had a significant effect on financial burden. Financial burden, in turn, had a significant effect on postponing care. Further, financial burden had both a direct and indirect effect through postponing care on physical quality of life (QOL), and both a direct and indirect effect through postponing care on emotional QOL. This highlights a significant effect of financial burden on postponing care and poorer QOL among myeloma patients. [17]

In the case of hypertrophic cardiomyopathy (HCM), we learned that prior authorization, which may lead to coverage denials or delays, can be especially risky given the possibility of sudden cardiac death or other worsening symptoms and complications. The prescription filling process for Camzyos was also noted to cause substantial confusion due to provider preferences for how to order the medication through a specialty pharmacy. The patient community recommends improved communication between providers and specialty pharmacies to allow for a streamlined and collaborative process.[18]

Delays or denials for coverage were also major themes among the atopic dermatitis community. According to a survey of 1,272 adults with atopic dermatitis and their caregivers, 50% (636 patients) experienced a delay or denial of coverage in the past 12 months, and only 68% of these patients were aware they could appeal an insurer's decision to deny coverage.[19] The majority (60%) of delays were due to prior authorization and half (50%) of denials were due to step therapy.[19] The most common wait time for delays was 4-7 days (32%) and the most common timeframe to appeal a coverage denial was 8-14 days (37%).[20] When asked about the impact of missing doses due to delay or denial of coverage, the largest proportion of respondents (18%) reported having an atopic dermatitis flare.[19] On a Likert scale of 1 being no impact, and 5 being significant impact, the majority (64%) of respondents reported the life impact of these delays and denials as a 3 or higher.

A 2021 study of the financial burden of atopic dermatitis found that OOP expenses included costs related to health care providers and prescriptions (visit deductibles, prescription co-payments, prescriptions not covered by insurance), nonprescription health care products, and complementary approaches to care and care coordination (e.g., specialized cleaning products, transportation, or parking fees).[21] Respondents (n=869) to this survey had a median annual OOP expense of $600 to manage their atopic dermatitis, and 41.9% (364 out of 869 patients) had OOP expenses of greater than $1,000.

In addition to the insights from the patient groups, ICER also looked to external sources of information to better understand the access challenges facing the patient community more broadly. In the 2023 Patient Experience Survey commissioned by Pharmaceutical Research and Manufacturers of America (PhRMA), more than 5,000 Americans weighed in to report on barriers to accessing health care and prescription drugs. Aligned with the comments we heard from all the patient groups, this survey found that 32% of respondents named OOP costs (e.g., copays, deductibles, coinsurance) as a top health care concern.[22] Furthermore, 19% of insured Americans reported that their current OOP expenses are more than they could afford in the case of an unexpected medical event and 53% reported that even with health insurance, they cannot anticipate the cost of their health care services.

As these data and the above patient group comments illustrate, many patients face access challenges that do not align with the ethical framework of providing patients with fair access to medicines. These examples are meant to highlight what is difficult to evaluate through our focus on insurance coverage policy documents.

6. Discussion

This assessment set out to evaluate whether coverage policies for drugs evaluated in ICER reviews during the calendar year 2021 were covered by major payers as of June 1, 2023 in concordance with fair access criteria for cost sharing, clinical eligibility criteria, step therapy, and prescriber restrictions. Exploratory analyses were also performed on the degree of transparency of policies regarding clinical coverage criteria, tiering, copay adjustment programs, and continuation of coverage for newly entering plan members. As noted in the introduction, our assessment was not able to evaluate many critical elements of how coverage policies are administered in the real world, including how efficient the prior authorization process is to clinicians and patients at the time of the clinical encounter, and how responsive payers are to requests for medically appropriate exceptions. These limitations are important in framing the results of the assessment, which found a high level of concordance of coverage policies with fair access criteria across large and small formularies of large private payers, the Veterans Health Administration, and state health insurance exchanges.

Despite the high overall level of concordance, non-concordance with cost sharing, clinical eligibility, and step therapy criteria was clustered in a few drugs or condition areas, including treatments for lupus nephritis (Benlysta), atopic dermatitis (Adbry), and myasthenia gravis (Soliris and Vyvgart).

As noted earlier, ten payers did not have Benlysta on the lowest relevant tier, and two payers listed Benlysta as non-formulary despite it being priced at a level that meets traditional cost-effective thresholds. Although Benlysta is more expensive than other first-line options, as noted earlier, we believe that it is more appropriate to steer patients to less-expensive options through step therapy than to use higher cost-sharing levels unless patients who try other options without adequate response can then obtain Benlysta at the lower out-of-pocket level. Drugs with higher cost sharing have been found to be associated with less adherence and higher discontinuation rates.[23] We believe therefore that placement of fairly-priced drugs such as Benlysta on preferred tiers increases adherence and provides incentives for manufacturers to price drugs according to value at launch, thus decreasing costs for both payers and patients.

Drugs for atopic dermatitis and myasthenia gravis were also more likely to have coverage that did not meet fair access criteria. Although 81% of formularies had clinical criteria concordant with fair access for Adbry, a drug for moderate-to-severe atopic dermatitis, those formularies lacking concordance often had overly narrow definitions of “moderate-to-severe” disease, with some formularies not allowing coverage for patients unless they have 10% body surface area (BSA) involvement. While it is consistent with clinical trial inclusion criteria to focus on BSA alone to define moderate to severe disease, this approach diverges from consensus guidelines, which include in the definition patients with lower overall BSA involvement but with disease in “critical areas” such as the face or feet that has a disproportionate impact on quality of life.[24]

In myasthenia gravis, several formularies required patients to step through more than three treatments before accessing Soliris and Vyvgart. Although these drugs are generally reserved for patients who have refractory disease, requirements to step through more than two treatments likely delays care for the sickest patients. Although step therapy is justifiable when used to encourage use of safer, better established treatment options, or the use of equally effective therapies at lower cost, a recent survey of US medical providers suggested that step therapy requirements may alter clinical decisions due to a desire to avoid prior authorization burdens, even in cases where the medication was clinically appropriate.[25] To address these concerns, some payers are implementing programs specifically to reduce prior authorization burden. For example, HCSC introduced a tool to streamline and accelerate the prior authorization process by automatically approving requests when certain criteria are met. During the pilot phase HCSC reported automated approvals were granted 66% of the time for specialty pharmacy decisions, with approvals being delivered almost instantaneously. This timing is considerably shorter compared to previous wait times of up to 14 days.[26] Plans should be applauded for these efforts, and it is critical they provide transparency into how these programs are structured and administered to show they are having the intended impacts. Similar-sounding programs have been implemented by other payers to speed up prior authorization, although recent news reports about coverage denial algorithms operating with minimal, if any, review by doctors, highlights ongoing concerns regarding how prior authorization criteria are implemented. [27]

In this year's report, we evaluated both the largest and smallest formularies for each large commercial payer, as determined by the number of covered lives, to examine whether size impacts rates of concordance with fair access criteria. However, given the high concordance rates across all formularies, and between health exchange formularies in different regions of the country we did not find substantial differences in fairness of coverage.

With the exception of our findings on cost sharing criteria, which were limited by the small number of drugs eligible for analysis, the results of this assessment suggest that the vast majority of coverage policies across these formularies are structured – on paper -- to provide fair access for this set of drugs. Insurers are sometimes criticized for interfering with decisions that should be made solely by patients and their clinicians, but all insurers have a responsibility to use evidence to establish prudent limits to coverage, and when structured appropriately and administered well, these policy tools can in many cases be important in protecting patients from the risks of care outside of established evidentiary boundaries. Moreover, it is important to recognize the financial stewardship that is delegated to payers in the US. Spending on health care is anticipated to continue to grow faster than the overall economy, leading to pressure on state and federal budgets as well as on the ability for employers and private payers to maintain affordable health insurance.[28] Increased spending on drugs is an important contributor to overall health care spending, lending ethical justification to the efforts by payers to use policies such as step therapy to address drug spending in ways that will not adversely affect patient outcomes.[29,30] In the main, our results

suggest that the payers in this assessment, in the design of their formularies, are meeting that challenge in a way that conforms with the broad outlines of criteria for fair access.

However, as noted throughout this report, there are important limitations to our analysis which should color any conclusions. Perhaps foremost, we were unable to evaluate many of the aspects of coverage policy implementation, such as the ease of obtaining medical exceptions, that are at the heart of many of the barriers experienced by clinicians and patients to appropriate coverage. It is also possible that the 19 formularies selected for this assessment provide coverage more consistent with fair access criteria than formularies from smaller payers, though our analysis did not show that the smallest formularies offered by large payers presented more barriers to access.

As noted earlier, our analysis may not reflect barriers to access created by implementation burdens on clinicians and patients. Our exploratory analyses last year in the 2022 Fair Access report found that prior authorization forms on a subset of drugs contained at least 22 – and as many as 71 – separate questions that providers were required to answer as part of prior authorization, a substantial administrative burden.[31] As noted earlier, news has emerged recently alleging that at least one national payer has instituted automated review systems that deny initial prior authorization requests according to algorithms, without direct medical review.[27] An analysis of the existing peer-reviewed and professional literature estimated that payers, manufacturers, physicians, and patients together incur approximately $93.3 billion in costs annually on implementing, contesting, and navigating utilization management. [32] Further, a small but growing number of employers and other plan sponsors are avoiding paying for expensive specialty drugs by excluding them entirely from the plan benefit.[33] These specialty carve-out or "alternative funding programs" are not the responsibility of insurers themselves but are recommended by some health benefit consultants to employers seeking to control their overall health insurance costs. The increasing use of these tactics is another important caveat to interpreting the generally favorable ratings in this year's report – when access to therapies is dictated not by transparent payer and PBM coverage policies, but by opaque specialty carve-out programs, fair access for patients is at risk and impossible to judge from an analysis looking only at insurer coverage policies.

Our exploratory analyses this year focused on transparency of various policies that could be of great importance to individuals considering enrolling in an insurance program. While we were able to find clinical eligibility criteria posted for the majority of drugs, this information was often found exclusively through the clinician portal of the payer website, which most potential enrollees might never think of accessing. Furthermore, policies were less available for continuation of coverage (36-64% of formularies) for a prospective enrollee. To achieve greater transparency, payers should not only post clinical eligibility criteria in the patient-facing areas of their website, but also have clear descriptions of medical exception *and* continuation of coverage policies for all current or potential insurance plan members.

Similarly, although information on tiering was available for the subset of drugs studied, payers did not always make it clear whether drugs were subject to copay adjustment programs. These adjustment programs can have a substantial impact on the out-of-pocket payments required of patients, so payers should inform prospective enrollees whether formularies may be part of a benefit design using these programs, and how to find out further information. Positive examples from our assessment included Kaiser Permanente and United Healthcare, both of whom had clear, detailed, and easily accessible descriptions about potential copay adjustments on their respective websites.

7. Conclusion

This is ICER's third annual assessment of how well major insurers' coverage policies for prescription drugs align with a set of fair access standards. Although with each report we have refined our criteria for evaluation and expanded analyses to try to capture the current state of insurance coverage for drugs in the US, the results of our assessment are useful in understanding how well written policies meet fair access criteria, but our results also continue to be a reflection on the limitations of the evidence available to us. Our concordance ratings cannot fully capture patient and clinician lived experiences with aspects of coverage such as the real-world burden of cost-sharing and prior authorization on patients. We must also acknowledge that our judgments about cost-sharing for specialty drugs based on formulary tier placement are unable to capture the complexity of a system that offers multiple ways to combine deductibles, tiering, and cost sharing levels, all of which are ultimately selected by the plan sponsor and not the insurer. Context is critical yet our ability to see through the complexity and opacity of these systems is limited.

However, we believe that these limitations should not take away from some important themes highlighted in this report. Payers should be given credit for generally structuring formularies to support many key elements of fair access. As with our prior Barriers to Fair Access reports, changes in coverage policies noted following the initial assessment show that payers are listening and engaged in continually assessing coverage processes and policies, which can lead to positive change. Our reports continue to demonstrate that greater transparency is needed around not only coverage policies but specialty carve-out programs and out-of-pocket costs, particularly as programs such as copay accumulators become more common. Some payers have taken steps to increase transparency by posting all their policies publicly; others hold coverage policies and tiering in confidence, perhaps to seek advantages against the competition. However, only with greater transparency across the industry will payers be able to demonstrate their commitment to the appropriate application of evidence to insurance coverage. And only with greater transparency will payers' call for fair pricing be heard by the public with the power it deserves.

In closing, this assessment is not meant to produce a definitive evaluation of fair access for pharmaceuticals; however, we hope that it continues to help move all participants in the health system toward greater understanding and dialogue. We wish to note again that underlying this effort is the white paper on Cornerstones for Fair Access that was produced with substantial guidance and input from members of the ICER Policy Leadership Forum. We wish to acknowledge and thank the participants in that effort, and those individuals who gave us continued input as part of our Working Group for this assessment. None of these individuals, or organizations, should be viewed as agreeing with this assessment, and any errors in this paper are solely the responsibility of the authors. To all, however, we give our thanks and our praise for their honesty and willingness to pursue a common goal from different starting points.

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