Assessment of Barriers to Fair Access

Final Report

December 19, 2024

Institute for Clinical and Economic Review

AUTHORS:

Grace A. Lin, MD
Medical Director for Health Technology Assessment
Institute for Clinical and Economic Review

Max Lee, PharmD
Pharmaceutical Intelligence Manager
Institute for Clinical and Economic Review

Belén Herce-Hagiwara, BA
Senior Research Assistant
Institute for Clinical and Economic Review

Dmitriy Nikitin, MSPH
Senior Research Lead, Evidence Synthesis
Institute for Clinical and Economic Review

Catherine Koola Fischer, MPH
Director, Patient Engagement
Institute for Clinical and Economic Review

Meaghan Cummings, MBA
Special Assistant to the President
Institute for Clinical and Economic Review

Sarah K. Emond, MPP
President and Chief Executive Officer
Institute for Clinical and Economic Review

DATE OF PUBLICATION: December 19, 2024

How to cite this document: Lin GA, Lee M, Herce-Hagiwara B, Nikitin D, Fischer CK, Cummings M, Emond SK,. Assessment of Barriers to Fair Access. Institute for Clinical and Economic Review, December 19, 2024. https://icer.org/assessment/fair-access-coverage-policies-in-2024/

About ICER

The Institute for Clinical and Economic Review (ICER) is an independent, non-profit research institute that conducts evidence-based reviews of health care interventions, including prescription drugs, other treatments, and diagnostic tests. In collaboration with patients, clinical experts, and other key stakeholders, ICER analyzes the available evidence on the benefits and risks of these interventions to measure their value and suggest fair prices. ICER also regularly reports on the barriers to care for patients and recommends solutions to ensure fair access to prescription drugs. For more information about ICER, please visit www.icer.org.

The funding for this report comes from non-profit foundations, with the largest single funder being the Arnold Ventures. No funding for this work comes from health insurers, pharmacy benefit managers (PBMs), or life science companies. ICER receives approximately 23% of its overall revenue from these health industry organizations to run a separate Policy Summit program, with funding approximately equally split between insurers/PBMs and life science companies. Life science companies relevant to this review who participate in this program include: Blue Shield of California (Blue Shield of CA), CVS Health, Elevance, Express Scripts, Health Care Service Corporation (HCSC), Kaiser Permanente, and UnitedHealthcare. For a complete list of funders and for more information on ICER's support, please visit https://icer.org/who-we-are/independent-funding/.

Table of Contents

List of Acronyms and Abbreviations Used in this Report

AAV	Adeno-associated virus
ALS	Amyotrophic lateral sclerosis
ALSA	ALS Association
ALSFRS-R	ALS Functional Rating Score Revised
ASP	Average sales price
AWP	Average wholesale price
BCBS	Blue Cross Blue Shield
Blue Shield of CA	Blue Shield of California
BMI	Body Mass Index
evLYG	Equal value of life years gained
FDA	US Food and Drug Administration
FSS	Federal Supply Schedule
HBPB	Health benefit price benchmark
HCSC	Health Care Service Corporation
HMO	Health Maintenance Organization
ICER	Institute for Clinical and Economic Review
OAC	Obesity Action Coalition
PBM	Pharmacy benefit manager
Prior Auth	Prior authorization
QALY	Quality-adjusted life year
SGLT-2i	Sodium-glucose cotransporter 2 inhibitors
US	United States
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 11 drugs reviewed by ICER in 2022: Mounjaro for Type 2 diabetes; Wegovy, Saxenda, Qsymia, and Contrave for obesity management; Radicava ORS for amyotrophic lateral sclerosis; Cosela for chemotherapy-induced neutropenia; Veozah for vasomotor symptoms of menopause; Zynteglo for beta thalassemia; Roctavian for hemophilia A; and Hemgenix for hemophilia B. ICER has elected not to include agents for treating COVID-19 that were reviewed in 2022 as those drugs were part of a "special assessment" and not subject to a traditional ICER review, and because the treatment landscape for COVID-19 has evolved significantly since 2022.

We assessed coverage policies for the selected drugs across 11 formularies, including the largest formularies by number of covered lives offered by the 10 largest commercial payers in the United States (US) and the single formulary of the Veterans Health Administration (VHA). At the time we conducted our research, these formularies represented coverage policies governing pharmaceutical access for approximately 57 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 a subset of requirements of ICER's fair access criteria. We defined concordance as the alignment of the coverage policy with ICER's criteria in four areas: 1) cost sharing to patients, with a single criterion requiring that a fairly priced drug 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.

New to this year's report, we conducted exploratory analyses of consumer accessibility of the drugs in scope and obtained at the pharmacy using data from IQVIA's Market Access Library solution. We

report data on proportion of prescriptions filled, rejected, abandoned, or paid in cash, and out-of-pocket costs. We also conducted additional exploratory analyses of whether, for each formulary, payers provide individuals shopping for health insurance sufficient transparency into cost sharing and tiering structure, and clinical eligibility criteria. These exploratory analyses were conducted looking for information on the three gene therapies being reviewed this year, Zynteglo, Roctavian, and Hemgenix.

Our analyses found overall high concordance rates across all formularies and the four criteria assessed. Specifically, we found:

- There was 100% concordance across formularies for step therapy and provider restrictions. Concordance for clinical eligibility was 96%.
- Overall concordance for cost sharing was 81% for the three drugs (Mounjaro, Wegovy, and Qsymia) that met ICER's definition of being fairly priced for long-term value.
  - Concordance was 100% for Mounjaro, as all formularies placed this drug or equivalent on the lowest relevant tier.
  - Concordance was 70% for both Wegovy and Qsymia, since some formularies did not place either the drug or an equivalent on the lowest relevant tier.

While the results of this assessment suggest that the vast majority of analyzed coverage policies across these formularies are structured – on paper -- to provide fair access for this set of drugs, high concordance may not necessarily translate into real-world access, defined as the process between a prescription being written and a patient obtaining the medication. For example, a payer may have a written policy for a drug, but a plan sponsor could choose to exclude that drug, as is the case for many obesity medications. Furthermore, our exploratory analysis of consumer accessibility (filled prescriptions and out-of-pocket costs) suggests that fill rates of new-to-brand prescriptions varied across drugs and therapeutic areas. In particular,

- Obesity medications had a non-trivial (5-24%) level of abandonment (meaning the patient A substantial number of prescriptions (30-52%) for obesity medications were rejected due to non-coverage.
- did not receive their prescription), and also had the greatest volume of cash pay for prescriptions of the drugs for which we were able to obtain data.

We assessed the transparency of information for prospective plan members for Zynteglo, Roctavian, and Hemgenix as part of our transparency analysis. We found that while most plans posted clinical eligibility criteria, information on tiering and cost sharing was not widespread.

There are several important limitations to these findings:

- 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 copay versus co-insurance are often decided or influenced by the plan sponsor and not the administrator or PBM.
- We were 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.
- Our analysis of coverage policies did not take into consideration other factors that may influence coverage decisions. For example, even if obesity medications offer long-term value, plans and plan sponsors may consider limitations or exclusions on coverage due to the enormous potential budget impact of coverage.
- While data from IQVIA provided important insights into consumer accessibility of drugs at the point-of-sale, these data were only reported in the aggregate across all commercial payers, so we are unable to interpret the data in the context of specific plan policies.

The final section of our assessment presents input received from patient organizations regarding barriers to fair access that their members are experiencing. Although the experiences conveyed rely 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.

While the evidence available and the limitations of our research effort leave many questions, our results demonstrate that the majority of evaluated payer policies in the formularies included in our analysis are structured in a way to support many key elements of fair access. However, the policies are just part of the equation; barriers created by the implementation of the policies may hinder access and delay patient care. As well, payers are challenged by the potential budget impact of current and future drug costs and need to consider tradeoffs between an ideal level of coverage and keeping costs at a reasonable level for plan sponsors and patients. This is especially acute for purchasers considering coverage of the obesity medications. This year's exploratory analyses using IQVIA pharmacy data, transparency analyses, and collection of patient surveys and stories attempt to fill in some of the blanks but without data that are tied to plan-specific policies, it is impossible to draw conclusions about specific barriers to 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 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, adding an exploratory analysis related to the transparency of coverage criteria for prospective enrollees. In the 2023 report, we modified our methods to expand the set of payer formularies evaluated within both the commercial and health exchange markets. We also added new elements related to the transparency analysis, including whether payers make details regarding continuation of coverage (i.e., "grandfathering") and copay adjustment programs (e.g., copay accumulators, maximizers) publicly available.

Based on the experience with the first three reports, and with ongoing input from our multi-stakeholder Working Group, our 2024 report has been modified from prior reports as summarized below. These updates leave the basic approach largely consistent with the 2023 report. We asked each payer with formularies included in the scope to provide coverage policy information to us; and we leveraged the MMIT Analytics Market Access Database to locate any policies not provided by payers. This year we are assessing coverage for 11 drugs reviewed by ICER in 2022 that are currently

FDA approved for an indication consistent with the ICER review. One drug reviewed by ICER in 2022, plinabulin (BeyondSpring Inc.) for prevention of chemotherapy-induced neutropenia, received a complete response letter from the FDA and has yet to gain approval. Another drug, AMX0035 (Relyvrio™, Amylyx Pharmaceuticals) for amyotrophic lateral sclerosis, was granted FDA approval but a subsequent failed readout from the Phase 3 trial prompted the manufacturer to withdraw the product from the market. As such, both drugs are excluded from the report. In addition, the agents for treating COVID-19 that were reviewed in 2022 are not included in this analysis as those drugs were part of a "special assessment" and not subject to a traditional ICER review, and because the treatment landscape for COVID-19 has evolved significantly since 2022.

We assessed coverage policies for the selected drugs across 11 formularies. We identified the 10 largest commercial payers in the US and selected their largest formulary by number of covered lives, based information from the MMIT Analytics Market Access Database. We also included the single formulary of the Veterans Health Administration (VHA). At the time we conducted our research, these formularies represented coverage policies governing pharmaceutical access for approximately 57 million Americans.

New for this year, ICER has partnered with IQVIA, a leading healthcare data and analytics provider, to gain insights into national level cost sharing and prior authorization metrics from real-world claims data. Based on insights licensed from IQVIA's Market Access Analytic Solutions, we evaluated measures illustrating average *'consumer accessibility'* (ability to obtain the drug at the pharmacy and out-of-pocket cost to obtain the drug) for a subset of the 11 drugs in scope for the past two years in the commercial line of business.

As with previous years, we included an evaluation of fair access criteria from the 2020 white paper related to the transparency of cost sharing (i.e. tiering) and of clinical eligibility criteria for a prospective plan enrollee. We focused this report's transparency analysis on the three gene therapies in scope this year: Zynteglo (beta thalassemia), Hemgenix (hemophilia B), and Roctavian (hemophilia A). These one-time therapies were selected based on their notable introduction into standard clinical practice, their potential economic impact, and greater likelihood that prospective enrollees would consider the availability of such novel therapeutic modalities as a determinant in their health plan selection process.

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 by payers and plan sponsors. The addition of the new IQVIA Market Access data analysis in this year's report serves to address some of these concerns; however, the data obtained from IQVIA are aggregate data from all commercial payers, so our analyses do not directly reflect the specific policies of the payers and formularies evaluated in the report. 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, or public payers, around the country. Finally, we analyzed specific plans and policies for the drugs in scope and this may not fully reflect the all the challenges – including budgetary challenges – to providing fair access.

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. a. The out-of-pocket cost data obtained from IQVIA are aggregate data from all commercial plans so may not necessarily reflect the specific cost-sharing policies of the plans evaluated in this report.
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: US 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, one clinical specialty society, one clinical expert, one from a pharmacy benefit manager, one from a health plan, one from a benefit consultancy, and two 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; advised on the interpretation of the IQVIA data; and commented on important nuances

in the evaluation 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 11 drugs that are in scope for this year's report are shown in Table 2 below. Average net prices for these drugs between January 2023 and December 2023 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 indicates the drugs 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.

Table 2. Drug List

Brand Drug Name	Generic Drug Name	Indication	ICER Health Benefit Price Benchmark ǂ	Annual Net Price Estimated Above or Below ICER HBPB*
Mounjaro™	Tirzepatide	Diabetes: Type 2	$5,833	Below
Wegovy®	Semaglutide	Obesity Management	$10,029	Below
Qsymia®	Phentermine/ Topiramate	Obesity Management	$4,912	Below
Saxenda®	Liraglutide	Obesity Management	$4,912	Above
Contrave®	Naltrexone/Bupropion	Obesity Management	$2,456	Above
Cosela™	Trilaciclib	Chemotherapy-Induced Neutropenia	$512 per vial	Above
Veozah™	Fezolinetant	Menopause: Vasomotor Symptoms	$2,661	Above
Radicava ORS®	Oral Edaravone	Amyotrophic Lateral Sclerosis	$3,275	Above
Zynteglo™	Betibeglogene autotemcel	Beta Thalassemia	$2,497,082 per administration	Above
Hemgenix®	Etranacogene dezaparvovec	Hemophilia B	$3,027,200 per administration	Above
Roctavian™	Valoctocogene roxaparvovec	Hemophilia A	$2,006,876 per administration	Above

HBPB: health benefit price benchmark

* Average prices net of all discounts and rebates, for the year of 2023, obtained from SSR Health. For prices not available or deemed unreliable, prices are taken from the Federal Supply Schedule (FSS). For physician administered drugs we use the average sales price (ASP) plus 6%, if available.

ǂ ICER health benefit price benchmarks for the higher of the $150,000 per QALY or $150,000 per evLYG threshold, inflated to 2023 prices.

For these 11 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 11 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 57 million Americans (MMIT Analytics as of 08/01/2024). See Table A4.1 in the Supplement for detailed information on covered lives per formulary.

Table 3. Largest Formulary Offered by Each of the 10 Largest Commercial Payers and the VHA

Payer	Formulary Name	Plan Type	Tiers Available
CVS Health (Aetna)	CVS Caremark Performance Standard Control w/Advanced Specialty Control	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Generic or Non-Preferred Brand
Express Scripts PBM	Express Scripts National Preferred	Commercial	1 - Formulary Generics 2 – Formulary Brands 3 – Non-formulary Brands
UnitedHealth Group, Inc.	UnitedHealthcare Advantage 3-Tier	Commercial	1 – Lower-cost (preferred generics and brand) 2 – Mid-range cost (preferred brand) 3 – Highest-cost (non-preferred brand and generics)
Cigna Corporation	Cigna Standard Three Tier	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Brand
OptumRx	OptumRx Premium Formulary	Commercial	1 – Lower-cost (preferred generics and brand) 2 – Mid-range cost (preferred brand) 3 – Highest-cost (non-preferred brand and generics)
Kaiser Foundation Health Plans, Inc.	Kaiser Permanente Southern California 3 Tier HMO	Commercial	1 – Most Generic drugs 2 – Most Brand-name drugs 3 – High-cost Brand-name or Generic drugs
Elevance Health, Inc.	Anthem Essential 4 Tier	Commercial	1 – Lower-cost (Preferred Generics) 2 – Mid-cost (Preferred Brand) 3 – High-cost (Non-Preferred Brand and Generics) 4 – Highest-cost (Specialty)
Health Care Service Corporation (HCSC)	BCBS of Illinois Basic 6 Tier	Commercial	1 - Preferred Generic 2 - Non-Preferred Generic 3 - Preferred Brand 4 - Non-Preferred Brand 5 - Preferred Specialty 6 - Non-Preferred Specialty
Highmark, Inc.	Highmark Blue Cross Blue Shield 3 Tier	Commercial	1 - Generic 2 - Preferred Brand 3 - Non-Preferred Brand
Blue Shield of California (Blue Shield of CA)	Blue Shield California Plus Formulary	Commercial	1 - Generic/Low-cost 2 - Preferred Brand 3 - Non-Preferred Brand 4 - Specialty/High Cost
Veterans' Health Administration (VHA)	VHA National Formulary	Federal	The VHA has three categories: formulary, formulary with prior authorization, and non-formulary but covered with clinical justification. There is a flat cost-sharing regardless of the category.

BCBS: Blue Cross Blue Shield, HMO: Health Maintenance Organization, PBM: Pharmacy Benefit Manager, VHA: Veterans Health Administration

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 11 drugs that were the subject of ICER evidence reviews in 2022 and are currently FDA approved for the indication for which ICER evaluated them. We defined concordance as the alignment of the coverage policy with ICER's criteria in four areas: 1) cost sharing to patients; 2) clinical eligibility; 3) step therapy policies; and 4) provider qualification restrictions. 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."

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 or who leave benefit plan type up to the determination of the plan sponsor. 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 (as determined by its price being below the $150,000 per evLYG threshold) 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, there are limitations to this approach. Payers noted that four-tier formularies are designed by request from plan sponsors to put all specialty drugs on the fourth tier, regardless of the drug's value. 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 the Kaiser Family Foundation 2024 Employer Health Benefits Survey, employees in non-high deductible plans were more likely to have plans with four or more tiers (61%) than three tiers (28%).[1] The same report also noted that, despite a higher percentage of four-tier formularies requiring co-insurance compared with three-tier formularies (41% to 30%), the most common form of cost sharing for workers covered by plans with three or more tiers is co-payment, not co-insurance.[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 for the member for a period of time and, where 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 as employers and other payers do not see their costs dramatically reduced, and ultimately increasing the costs of pharmaceutical coverage.[2] Benefit designs using either copay maximizer or accumulator mechanisms represent another important limitation in our ability to use formulary tiering as a surrogate for the cost sharing burden borne by patients.

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 (i.e., drugs priced at or below ICER's

HBPB) should be placed on the lowest available relevant tier, which for brand name drugs is the preferred brand tier (usually the second tier).

Methods for Assessment of Patient Cost Sharing and Accessibility

To evaluate drug accessibility and cost of the drugs in scope at the consumer level, we obtained data from IQVIA Longitudinal Access and Adjudication Data database, part of the IQVIA Market Access Analytic Library. This database reports information on prescriptions written and/or dispensed at a US pharmacy, including patient out-of-pocket costs. The data from IQVIA obtained by ICER includes data from calendar years 2022 and 2023 from all commercial third-party payers, excluding Medicare and Medicaid. We also obtained data on prescriptions that were paid in cash by the patient (patient may have been uninsured or insured but chose to not use their insurance benefits). We excluded the three gene therapies in scope (Hemgenix, Zynteglo, Roctavian) from this analysis since they are typically covered under the medical benefit and thus not available through outpatient pharmacies.

For each drug, we obtained data to assess patient access at the point-of-sale, including the potential burden of prior authorization, and patient cost-sharing measures. The data points provided by IQVIA included:

Patient accessibility measures

- Total written commercial prescriptions
- Total dispensed commercial prescriptions
- Total dispensed New to Brand commercial prescriptions
    - % filled on first attempt
    - % filled after multiple attempts
    - % of prescriptions rejected
        - % rejections due to prior authorization or step therapy
        - % rejections due to another reason (e.g., not covered, fill limit, etc.)
        - % abandonment of prescription (i.e., prescription was not picked up by patient and thus fill was reversed and drug was restocked; also called "reversal").

Patient cost-sharing measures

- % of prescriptions by out-of-pocket cost, ranges
- %/# cash pay prescriptions
    - Amount of cash pay transaction, in categories

Prescription claim status was determined after a 30-day look forward period; all data were normalized to a 30-day supply of the drug. All measures are reported in the aggregate; we do not report any identifiable information at the payer or plan level.

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 any 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
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: US 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: US 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

In this year's report, we revised our transparency analysis to focus on gene therapies, evaluating the extent to which formulary tiering and clinical eligibility information is available to consumers prior to health plan selection. We examined public-facing website information for each formulary to assess how easily prospective plan enrollees could find details on cost sharing/tiering, clinical criteria, and site of care. This process aimed to replicate the experience of someone seeking information on three one-time gene therapies: Zynteglo, Roctavian, and Hemgenix. Due to the complex logistics between manufacturers and health plans for these treatments, we also reviewed the customer-facing websites of the respective gene therapy manufacturers.

4. Results

We evaluated coverage policies for 11 drugs across 11 formularies for a maximum of 121 possible drug-formulary policy combinations. In each category of fair access, some criteria were not applicable, either because the drug was not covered, the drug was not priced cost-effectively (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 11 drugs were provided by the majority of payers. The MMIT database of coverage policies was used to supplement tiering information for one payer (Kaiser). 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 or excluded from coverage. Although plans allow coverage of non-formulary agents through a medical exception process, only drug-specific policies were evaluated for this report. Due to the large potential budget impact, some payers exclude the obesity medications in scope (Wegovy, Saxenda, Qsymia, Contrave) from coverage, but provide the option for groups to opt-in for obesity medications. For this assessment, we evaluated the policies provided by these payers on all criteria as if the agents are a covered benefit, in order to assess for fair coverage in cases where obesity medications are covered. Coverage policies for the gene therapies in scope (Zynteglo, Roctavian, Hemgenix) provided by pharmacy benefit managers (PBMs) were not included in our concordance assessment as these policies are decided and adjudicated by health plans.

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 9 below, overall results for concordance with the four fair access criteria domains measured range from a low of 81% for cost sharing, to a high of 100% for step therapy and prescriber restrictions.

Table 9. 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	31/33 (94%)	25/31 (81%)
Clinical eligibility	97/97 (100%)	93/97 (96%)
Step therapy	97/97 (100%)	97/97 (100%)
Prescriber restrictions	97/97 (100%)	97/97 (100%)

The percentage of policies judged concordant in Table 9 above uses the number of applicable 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 10 below presents the results with not applicable drug policies as a component of all policies evaluated.

Table 10. Overall Rate of Concordance with Fair Access Criteria Assessed

Fair Access Criterion	Concordant n (%)	Not Concordant n (%)	Not Applicable* n (%)
Cost sharing	25 (21%)	6 (5%)	90 (74%)
Clinical eligibility	93 (77%)	4 (3%)	24 (20%)
Step therapy	97 (80%)	0 (0%)	24 (20%)
Prescriber restrictions	97 (80%)	0 (0%)	24 (20%)

*Not applicable includes cases when the drug is not covered by the payer. 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

Three drugs out of the 11 were determined to be priced, net of rebates and discounts, within reasonable cost-effectiveness levels: Mounjaro, Wegovy, and Qsymia.

Mounjaro had a 100% (10/10) concordance rate for cost sharing, with all payers placing it on their lowest relevant tier.

Wegovy had a 70% (7/10) concordance rate for cost sharing. Three formularies (HCSC, Highmark, UnitedHealth) placed the drug on a non-preferred brand tier, with no other drugs in its class covered at the lowest relevant tier. With the input of clinicians and consistent with clinical guideline recommendations, we determined Wegovy to be part of the class of obesity medications that also includes Saxenda, Qsymia, Contrave, and Zepbound. Although some doctors may consider prescribing phentermine, we did not consider phentermine alone to be part of the class as it is not FDA approved for long-term use, and some clinical experts did not believe it to be equivalent to the obesity medications in our scope. We also did not consider non-pharmacologic interventions (such as lifestyle management) to be part of the class.

Qsymia had a 73% (8/11) concordance rate for cost sharing, with three formularies (HCSC, Highmark, UnitedHealth) listing the drug as a non-preferred brand, with no other drugs in its class covered at the lowest relevant tier. We did not consider the generic components of Qsymia (phentermine, topiramate) to be a part of the same drug class as Qsymia as phentermine is not approved for long-term use and Qsymia's dosages are not able to be replicated by prescribing the two generic components separately. As stated above, we also did not consider phentermine or non-pharmacologic interventions (such as lifestyle management) to be part of the class of obesity medications.

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. Of the assessed drug/payer combinations, 93/97 (96%) were concordant. There were four drug-payer pairs that did not meet fair access criteria.

Qsymia: One payer (Blue Shield of CA) required that a patient has not undergone bariatric surgery within 12 months of receiving Qsymia. This does not meet our criteria as it is not part of the labeled indication or clinical guidelines. (The concordance ratings are as of July 12, 2024; see "Changes to Payer Coverage Policies After Draft Analysis" section below for updated information).

Mounjaro: One payer (VHA) required that individuals have inadequate glycemic control on at least 1 mg of semaglutide injection plus two or more glucose lowering drugs (metformin, empagliflozin, insulin, pioglitazone, sulfonylurea) for at least six months and that the change needed to achieve goal A1C is less than 1%. This does not meet our criteria because these requirements are more restrictive than clinical guidelines.

Saxenda: One payer (VHA) implemented restrictive criteria requiring individuals to have an inadequate response to an agent which is not recommended in the clinical guidelines for the treatment of obesity. This requirement does not align with our criteria as the current clinical guidelines do not recommend the use of orlistat for obesity management.

Wegovy: One payer (VHA) established additional requirements, including documentation that prior VA National Formulary agents for weight management (e.g., orlistat) were not tolerated or deemed inadequate, as well as specific BMI criteria and conditions related to Type 2 diabetes. These requirements do not align with our clinical eligibility criteria since current clinical guidelines do not recommend the use of orlistat for obesity management, and the additional segmentation of BMI categories exceed the FDA label and clinical guidelines.

3. Step Therapy

Of the assessed drug/payer combinations, 97/97 (100%) were concordant with the fair access criteria for step therapy, meaning no policy required more than three steps for coverage and all step therapies were clinically appropriate.

As shown in Table 11 below, although concordance was 100% for our step therapy criteria across all formularies, we found some variation within drugs in the number of steps required before receiving coverage.

Table 11. Number of Steps Required for Prior Authorization by Drug

Drug Brand Name (Formulary type)	Most Common # of Steps	Range	Payers With the Highest Number of Steps Required
Mounjaro	0, 1	0-3	VHA
Wegovy	0	0-1	VHA
Qsymia	0	0	N/A
Saxenda	0	0-2	VHA
Contrave	0	0	N/A
Cosela	0	0	N/A
Zynteglo	0	0	N/A
Radicava ORS	0	0-1	Express Scripts, HCSC, Blue Shield of CA
Hemgenix	0	0	N/A
Roctavian	0	0-1	UnitedHealth, Highmark
Veozah	2	1-3	Elevance

HCSC: Health Care Service Corporation, N/A: Not Applicable, VHA: Veterans Health Administration

4. Prescriber Restrictions

Of the assessed drug/payer combinations, 97/97 (100%) were concordant with the fair access criteria for prescriber restrictions either because written policies did not have prescriber restrictions or because requiring specialty prescribers was deemed appropriate for the condition. Written policies for the following drugs did not restrict coverage to specialty prescribers: Wegovy, Saxenda, Qsymia and Contrave for obesity management; Mounjaro for Type 2 diabetes; and Veozah for vasomotor symptoms of menopause. The following drugs had written policies requiring specialist prescribing for coverage that was deemed appropriate for the condition: an oncologist for the coverage of Cosela for chemotherapy-induced neutropenia, a hematologist for the coverage of Zynteglo for beta thalassemia, a neurologist for coverage of Radicava ORS for amyotrophic lateral sclerosis, a hematologist or hemophilia specialist for Hemgenix in hemophilia B and Roctavian in hemophilia A.

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"). When payers indicated that benefit plan type would be up to the determination of the plan sponsor, we assumed the plan type to be in line with the predominant plan type of other included policies. 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 in Table 12.

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 12. 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.

Drug (Indication)	Predominant Benefit Plan Type†	Cost Sharing	Clinical Eligibility	Step Therapy	Prescriber Restrictions
		Concordant Policies, n/N (%)*			
Mounjaro (Type 2 Diabetes)	Pharmacy (10/11)	10/10 (100)	10/11 (91)	11/11 (100)	11/11 (100)
Wegovy (Obesity Management)	Pharmacy (9/11)	7/10 (70)	10/11 (91)	11/11 (100)	11/11 (100)
Qsymia (Obesity Management)	Pharmacy (9/11)	8/11 (73)	9/10 (90)	10/10 (100)	10/10 (100)
Saxenda (Obesity Management)	Pharmacy (9/11)	N/A	10/11 (91)	11/11 (100)	11/11 (100)
Contrave (Obesity Management)	Pharmacy (7/11)	N/A	8/8 (100)	8/8 (100)	8/8 (100)
Cosela (Chemotherapy -Induced Neutropenia)	Medical (5/11)	N/A	7/7 (100)	7/7 (100)	7/7 (100)
Zynteglo (Beta Thalassemia)	Medical (8/11)	N/A	7/7 (100)	7/7 (100)	7/7 (100)
Radicava ORS (Amyotrophic Lateral Sclerosis)	Pharmacy (10/11)	N/A	10/10 (100)	10/10 (100)	10/10 (100)
Hemgenix (Hemophilia B)	Medical (8/11)	N/A	7/7 (100)	7/7 (100)	7/7 (100)
Roctavian (Hemophilia A)	Medical (9/11)	N/A	7/7 (100)	7/7 (100)	7/7 (100)
Veozah (Menopause: Vasomotor Symptoms)	Pharmacy (8/11)	N/A	8/8 (100)	8/8 (100)	8/8 (100)

N/A: Not applicable (meaning that these drugs are not priced at a cost-effective level (n=8) 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.

† Numbers in the parentheses are number of formularies with the predominant plan type/all formularies

Concordance by Payer

As shown in Table 13, the percent concordance across all 11 drugs on specific fair access criteria varies between formularies, ranging from 33% to 100% for cost sharing, 57% to 100% for clinical eligibility, and 100% for step therapy and prescriber restrictions. The non-concordance with cost sharing is driven mainly by some payers placing the obesity medications on a non-preferred tier. Additionally, the VHA tended to have strict criteria for clinical eligibility and 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 three, 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 11 drugs. In Table 13, 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 13. Rate of Concordance by Payer: Number (%) of Policies Meeting Each Fair Access Criterion out of all Relevant Policies

	Cost Sharing	Clinical Eligibility	Step Therapy	Prescriber Restrictions
Payer	*Concordant Policies, n/N* (%)*			
CVS Health (Aetna)	3/3 (100)	5/5 (100)	5/5 (100)	5/5 (100)
Express Scripts PBM	3/3 (100)	8/8 (100)	8/8 (100)	8/8 (100)
UnitedHealth Group, Inc.	1/3 (33)	11/11 (100)	11/11 (100)	11/11 (100)
Cigna Corporation	3/3 (100)	11/11 (100)	11/11 (100)	11/11 (100)
OptumRx	3/3 (100)	5/5 (100)	5/5 (100)	5/5 (100)
Kaiser Foundation Health Plans, Inc.	1/1 (100)	11/11 (100)	11/11 (100)	11/11 (100)
Elevance Health, Inc.	3/3 (100)	6/6 (100)	6/6 (100)	6/6 (100)
Health Care Service Corporation (HCSC)	1/3 (33)	11/11 (100)	11/11 (100)	11/11 (100)
Highmark, Inc.	1/3 (33)	11/11 (100)	11/11 (100)	11/11 (100)
Blue Shield of California (Blue Shield of CA)	3/3 (100)	10/11 (91)	11/11 (100)	11/11 (100)
VHA National Formulary (VHA)	3/3 (100)	4/7 (57)	7/7 (100)	7/7 (100)

PBM: pharmacy benefit manager

*The total N for each fair access criteria represents whether the specific criterion is applicable for that drug.

Patient Cost Sharing and Accessibility

In prior reports, health plan concordance rates with fair access criteria have been relatively high. However, feedback from patients and working group members suggested that those findings do not necessarily reflect real world access for patients, as prior authorization processes and patient out-of-pocket costs are not reflected in those concordance criteria. To supplement the core analyses of this report, we used information from IQVIA's Longitudinal Access and Adjudication Data from the IQVIA Market Access Analytic Library to examine aspects of consumer access for drugs in scope for all US commercial payers for the years 2022 and 2023. The IQVIA data offer insights into actual patient costs, as well as the rates of denials, prior authorization, and prescription abandonment.

Prescription Fill Data

Table 14 shows the proportion of prescriptions filled across all commercial payers, by drug and by year. There was substantial variation in successfully filled prescriptions on the first attempt. For example, a drug such as Mounjaro, indicated for Type 2 diabetes, had high success rates on the first attempt, likely reflecting widespread availability on formularies for this drug for this indication. However, there was a much lower fill rate after all attempts, which may reflect off-label prescribing for obesity (Zepbound did not gain FDA approval until late 2023) and fill attempts at multiple pharmacies due to drug shortages, among other reasons. On the other hand, obesity medications (Wegovy, Saxenda, Qsymia, Contrave) were filled at a much lower rate, with 15-23% of prescriptions successfully filled on the first attempt, and 18-37% filled after all attempts. For other drugs in scope, there were few prescriptions written and filled for Radicava for ALS, which would be expected given the rarity of the condition; similarly there were very few prescriptions written for Cosela, likely due to the fact that this drug is usually given with chemotherapy cycles, which are generally administered either in infusion centers or hospitals. Veozah fill rates were less than 50%; this may reflect that the drug did not receive FDA approval until mid-2023 and thus there may have been a lag between approval, establishment of coverage policies, widespread availability of the drug, and prescriber knowledge about a new treatment.

Table 14. Overall Commercial Consumer Prescription Accessibility, 2022 and 2023

Drug Name	Calendar Year	Total Written Prescriptions	Total New-to-Brand Dispensed	% Filled on First Attempt	% Filled after All Attempts*
Mounjaro	2022	1,536,182	444,393	74%	43%
	2023	8,712,178	959,308	59%	48%
Wegovy	2022	1,405,003	137,158	15%	21%
	2023	6,169,210	794,915	17%	26%
Saxenda	2022	908,705	161,122	23%	37%
	2023	826,347	127, 105	17%	31%
Qsymia	2022	127,629	16,027	22%	23%
	2023	158,295	22,925	22%	25%
Contrave	2022	165,224	20,931	18%	18%
	2023	227,205	39,161	22%	24%
Radicava ORS	2022	1,616	424	4%	15%
	2023	5,870	571	6%	10%
Radicava ORS Starter Kit	2022	421	367	--	7%
	2023	610	522	--	23%
Radicava	2022	149	10	--	4%
	2023	47	6	75%	60%
Veozah	2022	--	--	--	--
	2023	50,207	16,429	46%	42%
Cosela	2022	8	2	50%	50%
	2023	11	2	25%	50%

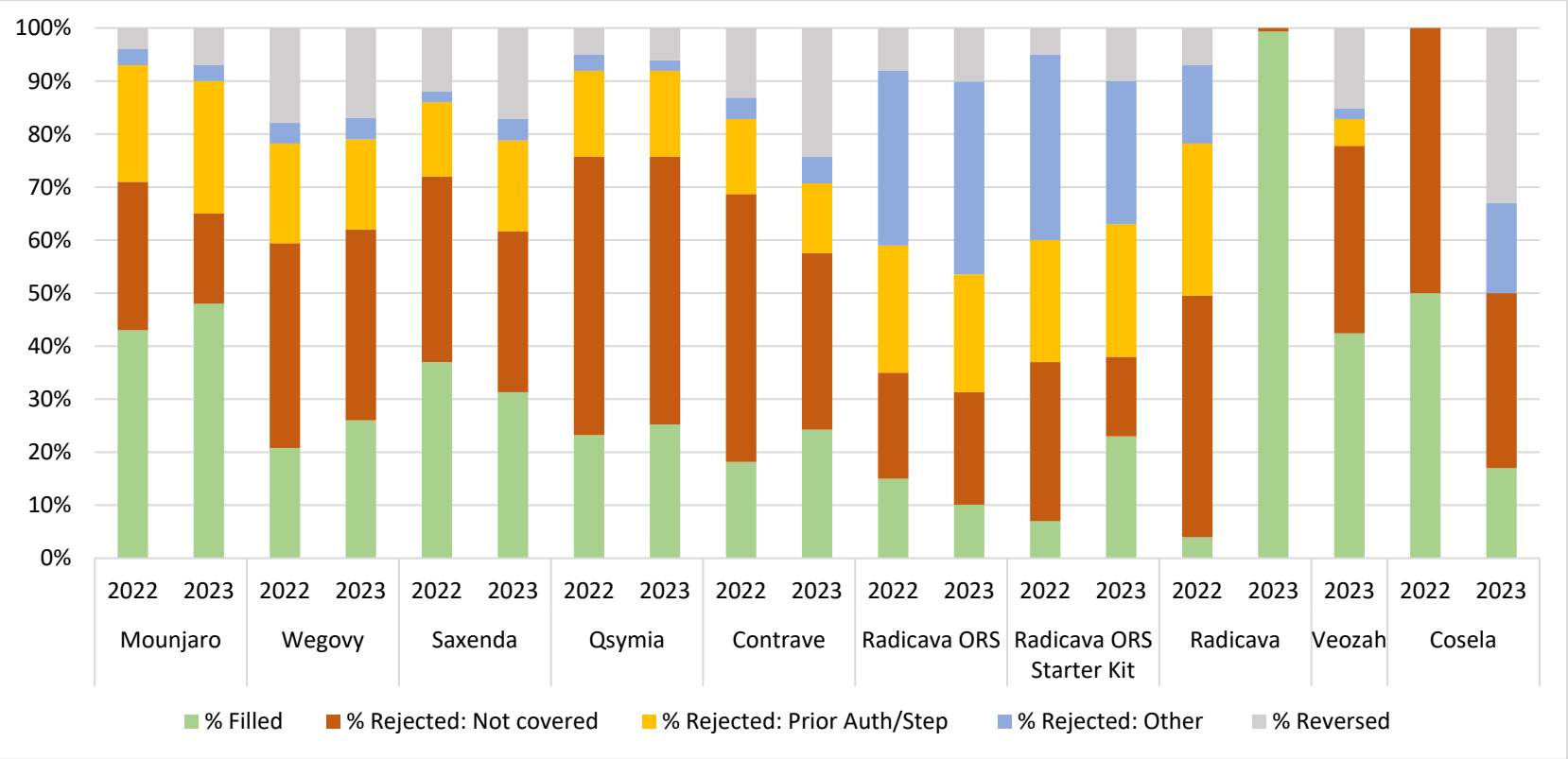
*The denominator includes any pharmacy-initiated attempts to fill the prescription, which may include test claims, administrative errors, pharmacists checking coverage or price of the drug, checking whether the drug has been approved, etc.

Figure 1 below shows the overall proportion of prescriptions filled after all attempts, as well as the reasons for rejection. The main reasons for rejection of a prescription are non-coverage of the drug, denial due to prior authorization or step therapy reasons, and other (e.g., administrative errors, quantity limits, plan limits, etc.). Finally, the proportion of abandoned prescriptions is also shown.

Many prescriptions were rejected due to non-coverage of the drugs; this was particularly true for the obesity medications, where 30-52% of rejections were for non-coverage. In contrast, only 12% of prescriptions of Mounjaro were rejected due to lack of coverage, likely reflecting widespread coverage for diabetes medications compared with more limited coverage for obesity. Non-coverage was also a frequent reason for rejection for Cosela, Radicava, and Veozah.

Rejections due to prior authorization or step therapy was less frequent, which would be consistent with many of these types of issues being resolved with multiple fill attempts. There was substantial variation in prescriptions for obesity medications that were eventually abandoned (5-24%), with overall higher abandonment rates in GLP-1 inhibitors compared with oral drugs. Of note, reasons for abandonment could include inability to obtain the medication due to drug shortages, as well as difficulty paying for a non-covered drug.

Figure 1. Rate of New to Brand Prescriptions Filled, Rejected, or Abandoned (Reversed) Across All Attempts – 2022 and 2023



Prior Auth: Prior Authorization; Step: Step Therapy

Out-of-pocket costs

We also obtained data on out-of-pocket costs, both for covered and cash prescriptions. This final out-of-pocket cost may include manufacturer copay assistance but would not reflect other kinds of discounts such as GoodRx coupons or debit cards provided by the manufacturer. Table 15 below shows the proportion of prescriptions sold for each category of out-of-pocket cost. For Mounjaro, Wegovy, and Saxenda, the majority of prescriptions fell between $20-30 out-of-pocket, which may reflect the impact of a manufacturer copay cards. Interestingly, out-of-pocket costs were higher for oral obesity medications such as Qsymia and Contrave, with 50% of claims falling somewhere between $50-250. For Radicava, the majority of prescriptions were sold for $0 out-of-pocket; however, a substantial minority were $1500 or more per prescription, particularly for the Radicava ORS Starter Kit or the Radicava infusion.

Table 15. Out-of-pocket Commercial Cost Per Prescription for New-to-Brand Prescriptions, by Drug and Amount Category

Drug Name	Calendar Year	Patient Out-Of-Pocket Commercial Final Cost; % of Total Normalized Claims (30-day)												
		$0	$0-10	$10-$20	$20-$30	$30-$40	$40-$50	$50-$75	$75-$125	$125-$250	$250-$500	$500-$1000	$1000-$1500	$1500+
Mounjaro	2022	12%	6%	2%	72%	2%	1%	1%	1%	1%	0%	1%	2%	0%
	2023	17%	9%	4%	50%	4%	3%	3%	2%	2%	1%	4%	1%	0%
Wegovy	2022	11%	1%	4%	66%	1%	1%	1%	0%	0%	1%	5%	5%	3%
	2023	23%	1%	4%	57%	1%	0%	1%	1%	0%	1%	4%	5%	2%
Saxenda	2022	12%	5%	7%	63%	1%	1%	1%	1%	1%	2%	2%	3%	1%
	2023	11%	5%	7%	58%	2%	1%	3%	2%	1%	2%	2%	4%	1%
Qsymia	2022	10%	1%	7%	7%	14%	10%	24%	13%	13%	0%	0%	0%	0%
	2023	11%	1%	7%	9%	15%	9%	24%	12%	12%	0%	0%	0%	0%
Contrave	2022	7%	3%	3%	8%	6%	6%	13%	11%	17%	14%	12%	0%	0%
	2023	6%	12%	3%	10%	3%	3%	7%	6%	32%	7%	12%	0%	0%
Radicava ORS	2022	71%	-	-	-	-	-	17%	4%	-	4%	4%	-	0%
	2023	74%	2%	5%	-	1%	1%	10%	1%	1%	1%	-	-	6%
Radicava ORS Starter Kit	2022	38%	0%	0%	-	-	-	25%	-	-	-	-	-	38%
	2023	55%	5%	9%	-	-	-	5%	-	-	5%	-	-	23%
Radicava	2022	50%	0%	16%	2%	9%	-	2%	3%	2%	-	-	3%	14%
	2023	55%	-	-	-	19%	-	-	-	26%	-	-	-	-
Veozah	2022	-	-	-	-	-	-	-	-	-	-	-	-	-
	2023	22%	1%	2%	2%	47%	4%	8%	5%	3%	1%	4%		0%

IQVIA did not report out-of-pocket metrics for Cosela and Hemgenix. Other products with no volume were not represented in IQVIA's data set and/or may not have been launched in the data time period. Darker color indicates higher percentage.

We note that out-of-pocket costs in Table 15 may vary based not only on formulary tiering but also by other benefit design structures. For example, for patients with high deductible health plans (estimated to be 29% of covered workers in 2023) out-of-pocket costs for a particular prescription fill will depend on whether they have met their deductible for the year.[3] This may account for the 2% of prescriptions that were in the $1000-$1500 category for Mounjaro, for example. Additionally, for patients with plans that include copay accumulators, manufacturer assistance programs like copay cards may or may not impact total out-of-pocket costs, since copay cards typically do not count towards annual deductibles or out-of-pocket maximums. The out-of-pocket costs may not reflect the full year patient experience as the data are focused on new-to-brand prescriptions only; out-of-pocket costs for subsequent refills may not be the same due to time-limited copay assistance, deductibles, and out-of-pocket maximums.

Cash Pay Prescriptions

Finally, we obtained data on the number of prescriptions that were paid entirely in cash for each drug in scope (Table 16). Cash prescriptions may reflect prescriptions that were submitted through insurance, rejected (e.g., for non-coverage), then the cost paid for in cash, or prescriptions that bypassed the insurance process entirely (e.g., if the patient already knew it was not covered and thus did not attempt to use insurance benefits). For New to Brand cash prescriptions, we also reported the monthly average wholesale price, as well as out-of-pocket cost range (Table 17).

Table 16. Overall Cash Pay Prescription Volume*, 2022 and 2023

Drug Name	Calendar Year	Total Cash Written Prescriptions	Total Cash New to Brand Dispensed
Mounjaro	2022	137,023	43,539
	2023	153,014	19,822
Wegovy	2022	33,774	2,258
	2023	82,270	13,117
Saxenda	2022	16,255	2,438
	2023	12,238	1,432
Qsymia	2022	3,906	830
	2023	4,468	1,055
Contrave	2022	26,617	7,995
	2023	47,673	14,607
Radicava ORS	2022	-	-
	2023	3	-
Radicava ORS Starter Kit	2022	-	-
	2023	3	-
Radicava	2022	74	6
	2023	1	-
Veozah	2022	-	-
	2023	861	267
Cosela	2022	-	-
	2023	1	-
Hemgenix	2022	-	-
	2023	-	-

*Metrics for cash claims may include double counting of denied commercial claims, so interpret with caution.

Table 17. Out-of-Pocket Costs for New to Brand Cash Prescriptions, by Drug and Amount Category

Drug Name	Monthly AWP (USD)*	Calendar Year	Patient Out-Of-Pocket Cash Final Cost; % of Total Normalized Claims (30-day)												
			$0	$0-10	$10-$20	$20-$30	$30-$40	$40-$50	$50-$75	$75-$125	$125-$250	$250-$500	$500-$1000	$1000-$1500	$1500+
Mounjaro	1,390	2022	-	-	-	-	-	-	-	-	-	-	1%	96%	3%
		2023	1%	-	-	-	-	-	-	-	-	1%	2%	89%	6%
Wegovy	1,754	2022	-	-	-	-	-	-	-	-	-	-	2%	1%	97%
		2023	-	-	-	-	-	-	-	-	-	-	1%	2%	95%
Saxenda	1,641	2022	-	-	-	-	-	-	-	-	1%	19%	17%	10%	52%
		2023	1%	-	-	-	-	-	-	-	1%	21%	19%	10%	48%
Qsymia	257	2022	-	-	-	-	1%	-	1%	24%	67%	5%	-	-	-
		2023	1%	-	1%	-	1%	-	1%	25%	61%	10%	-	-	-
Contrave	761	2022	1%	1%	1%	1%	1%	-	1%	1%	5%	20%	67%	-	-
		2023	1%	2%	3%	2%	1%	-	-	-	2%	6%	82%	-	-
Radicava ORS	18,354	2022	-	-	-	-	-	-	-	-	-	-	-	-	-
		2023	-	-	-	-	-	-	-	-	-	-	-	-	100%
Radicava ORS Starter Kit	N/A	2022	-	-	-	-	-	-	-	-	-	-	-	-	-
		2023	-	-	-	-	-	-	-	-	-	-	-	-	100%
Radicava	17,050	2022	-	-	-	-	-	-	-	-	-	-	-	50%	50%
		2023	-	-	-	-	-	-	-	-	-	-	-	-	-
Veozah	669	2022	-	-	-	-	-	-	-	-	-	-	-	-	-
		2023	-	-	-	-	-	-	-	-	-	2%	97%	-	-

AWP: average wholesale price

IQVIA did not report out-of-pocket metrics for Cosela and Hemgenix. Other products with no volume were not represented in IQVIA's data set and/or may not have been launched in the data time period. Darker color indicates higher percentage.

*Average prices net of all discounts and rebates, for the year of 2023, obtained from SSR Health. For prices not available or deemed unreliable, prices are taken from the Federal Supply Schedule (FSS). For physician administered drugs we will use the average sales price (ASP) plus 6%, if available.

The largest number of cash prescriptions were written for obesity medications. This is not unexpected, since many commercial plans do not cover drugs for obesity management or have stringent coverage criteria, due to the potentially large budget impact of covering this class of drugs. Cash prescriptions were most frequent with Wegovy and Contrave. There were also many cash prescriptions written for Mounjaro, which may reflect off-label prescriptions for obesity management instead diabetes, since Zepbound was not approved until November 2023. The out-of-pocket costs for cash were largely consistent with the calculated monthly average wholesale price (AWP) for each drug and in most cases this was in the hundreds to thousands of dollars per month.

There are several limitations to these analyses. First, the data provided are from all commercial payers for the years listed; we were not able to obtain payer-specific data and thus these data cannot be directly associated with the concordance results in our report. Additionally, the data reported are per prescription, and thus there may be multiple prescriptions for a single patient that may have been processed due to test claims, administrative errors, pharmacy shopping, or drug shortages. Finally, out-of-pocket costs do not account for certain discounts such as GoodRx coupons or manufacturer-provided debit cards. Additionally, since we analyzed new-to-brand prescriptions dispensed only, these data do not reflect out-of-pocket costs that may be incurred once manufacturer copay assistance ends, or the impact of copay accumulators and maximizers.

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, clinical eligibility criteria, and site of care.

Given the abundance of discussion surrounding the coverage of and eligibility for gene therapies, we selected these drugs for this year's transparency analysis. We used a targeted approach and elected to evaluate the availability of this information for Zynteglo, Hemgenix, and Roctavian.

Formularies offered by PBMs were excluded 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. Additionally, the Department of Veterans Affairs' formulary was excluded as the three drugs in scope for the transparency analysis are not applicable to their population. Following feedback from Kaiser, we decided to exclude them from the transparency analysis based on their description of the unique structure of their formulary, which indicates that non-formulary drugs are covered when a physician deems it medically appropriate for the patient. After excluding PBMs, Kaiser, and the VA, six formularies were assessed for transparency.

For each of these analyses, we conducted a search of the payer's website to determine whether information on clinical eligibility, cost sharing, and site of care 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. Summary results are presented in Table 18 below and ratings for each formulary / drug combination can be found in Supplement Tables B13.1-B13.3.

Table 18. Summary of Results for Exploratory Transparency Analyses

	Transparency of Cost Sharing and Tier Information	Transparency of Clinical Criteria	Transparency of Site of Care
Zynteglo	3/6 (50%)	5/6 (83%)	0/6 (0%)
Hemgenix	2/6 (33%)	5/6 (83%)	0/6(0%)
Roctavian	2/6 (33%)	5/6 (83%)	0/6 (0%)

Transparency of Cost Sharing

For cost sharing, a plan met criteria if they provided information showing the tiers for their formulary or clearly indicated the drug is not covered and the individual is responsible for the out-of-pocket cost of the drug. All gene therapies were included in this analysis, regardless of ICER's fair-pricing assessment. We found that two payers, Elevance and Cigna, provide cost-sharing details for Hemgenix and Roctavian while, three payers (Elevance, Highmark, and Cigna) provide publicly accessible information on cost sharing and tiering for Zynteglo.

We highlight information obtained from the Elevance and Cigna websites as examples where information related to the cost associated with each drug was presented to a potential patient shopping for a plan. Elevance clearly stated all three drugs (Zynteglo, Hemgenix, and Roctavian) were not covered and the full out-of-pocket responsibility would fall on the individual. Cigna also provided information regarding the cost sharing for each of the three gene therapies. However, the language for all drugs notes they are “non-preferred brand” and “excluded”. This language may be confusing to patients; further explanations about these designations were not found. Both payers were considered to meet our criteria as they present publicly available information related to the cost associated with each drug on their websites for a patient shopping for a plan.

Transparency of Clinical Criteria

A plan was considered to have transparent clinical criteria when a copy of the clinical policy for Zynteglo, Hemgenix or Roctavian was publicly accessible on the plan’s website without a login requirement. For all three drugs, five of the six plans had clinical policies publicly posted (Blue Shield of CA did not). Elevance stood out among the six plans as they provide access to their clinical criteria through the member section of their website for all three drugs within their Anthem Essential 4 Tier formulary.[4] In contrast, four of the plans (Cigna, HCSC, Highmark, UnitedHealth) posted clinical criteria policies only within the portion of the website intended for providers. It is possible that a prospective plan member would not explore the provider section of a website for materials, especially in some cases where a plan requires the user to attest that they are a health care provider.

Transparency of Site of Care

The administration of gene therapies requires specialized treatment centers; plans that included information on these sites of care (e.g., name of site, address, associated healthcare professionals) were assessed to be transparent on this criterion. These specialized treatment centers are often not evenly distributed across the country, potentially requiring many patients to travel out of state to access necessary care, which can pose additional barriers to treatment. This information was not found for any formulary-therapy combinations in our scope, even though manufacturer websites for all three gene therapies list sites of care. In acknowledgment of the limited availability of public information we recognize patients may collaborate with their provider and or a care team to navigate the process of receiving a gene therapy. However, it could streamline the process for potential plan members to have a point of contact (e.g. a phone number or email) for the plan in order to make inquiries. If any of the plans in scope had this type of information available on their public website, we would have found them to be concordant.

Changes to Payer Coverage Policies After Draft Analysis

Draft results of this analysis were shared with all payers on October 7, 2024 for a four-week comment period. During this period, payers were asked to submit comments and were invited to provide corrections, updates, and perspectives on draft concordance ratings. As part of the feedback received from payers, one payer informed us of changes to their policies affecting the obesity medications Qsymia and Wegovy. To preserve the integrity of the analysis, we have not included this change in the primary results presented above. However, 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 or no longer meet fair access criteria, we summarize changes in Table 19 below and present an updated overall concordance rating on clinical criteria for the affected formulary.

Table 19. Payer Policies Changes After July 12, 2024 That Would Have Resulted In Changes in Concordance Ratings with Fair Access Criteria

Formulary	Drug	Policy Change	Rating Before	Rating After	Payer Concordance with Policy Change
Changes to Clinical Eligibility Criteria					
Blue Shield of California Plus Formulary	Qsymia	Effective October 2024, criteria no longer exclude patients who have undergone bariatric surgery within 12 months of receiving Qsymia.	Non-concordant	Concordant	Clinical Eligibility Criteria 11/11 (100%)
Changes to Cost-Sharing					
Blue Shield of California Plus Formulary	Qsymia	Effective September 04, 2024, Qsymia's tier placement was changed from Tier 2 (Preferred Brand) to Tier 3 (Non-Preferred Brand). As such, Qsymia is no longer on the formulary's lowest relevant tier.	Concordant	Non-concordant	Cost-Sharing Criteria 1/3 (33%)
Blue Shield of California Plus Formulary	Wegovy	Due to the above change in Qsymia's tier placement, there are now no obesity medication in the same class as Wegovy available at the lower relevant tier.	Concordant	Non-concordant	Cost-Sharing Criteria 1/3 (33%)

5. Input from Patient Groups

To enhance our understanding of real-world patient experience with access to the therapies in scope for this year's report, we reached out to 21 disease-specific patient organizations across the eight therapeutic areas of interest. The following groups joined a call with ICER's Director of Patient Engagement and/or submitted comments and evidence to describe their community's experience with access challenges:

- ALS Association
- Coalition for Hemophilia B
- Cooley's Anemia Foundation
- Obesity Action Coalition
- Hemophilia Federation of America
- National Bleeding Disorder Foundation

The above groups have documented through 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.

Access to Radicava ORS for the ALS Community

A patient advocate from the ALS community helped collect insights from several people with ALS and their care partners regarding the range of experiences in accessing Radicava ORS in the United States. There was a mixture of people with ALS who were currently taking Radicava ORS, who chose not to take it, or could not afford it. The patients taking Radicava ORS received insurance coverage through a variety of payers including commercial plans, Medicare Part D, or a large public payer. Experiences with out-of-pocket costs, copay assistance, and coverage denials varied both across insurance plan types and sometimes within the same plan. Out-of-pocket costs for patients taking Radicava ORS ranged from $0-$1,400 a month. While all patients on the large public payer plan had a $0/month copay, we heard how patients had very different experiences even under the same commercial payer plan: 1) Copay assistance was not being applied to their deductible leading to a $1,400 monthly out-of-pocket expense, 2) Radicava ORS was not covered by insurance and required them to initiate an appeals process, and 3) Radicava ORS was covered and the process for approval was a positive experience. In contrast to the first example, some patients taking Radicava ORS shared that they did receive copay assistance from copay assistance foundations. Similarly, others shared they had minimal barriers to access Radicava ORS. Importantly, we also heard that some people with ALS chose to discontinue treatment due to cost.

These patient insights demonstrate the diversity of experiences with accessing Radicava ORS, both positive and negative, which is not apparent from the perfect concordance for clinical eligibility, step therapy, and prescriber restrictions as assessed in ICER's evaluation. Some of the variation in out-of-pocket costs for Radicava ORS was seen from the IQVIA data on patient cost sharing and accessibility; however, these patient stories add important context to those numbers.

The ALS Association (ALSA) shared that most people with ALS who have not served in the military purchase Medicare-Fee-for-Service or Medicare Advantage plans, which were not in scope for ICER's evaluation of coverage policies, and therefore might limit the complete picture of barriers to access to Radicava ORS. The ALS Focus Demographics Survey, developed and administered by ALSA, showed that among 528 people with ALS who completed the survey between 2020 to 2021, over two-thirds reported using Medicare, one in 10 Veterans Affairs, and approximately one in five private insurance for ALS care.[5] We heard that the devastating nature of ALS forces many patients out of the workforce, with the results of the Spring 2020 ALS Focus Survey showing that of the people with ALS who lost health insurance following ALS diagnosis, the majority (67%) of respondents lost their health insurance in part due to stopping employment or to provide ALS care.[6]

ALSA provided us with detailed information about the inconsistencies across payer policies for Radicava ORS and shared how narrowed clinical eligibility criteria has led to decreased access for people with ALS (see the Additional Patient Input section of the Supplement for further details). Although ICER's Fair Access Criteria allows for narrowed clinical eligibility in the case of a drug that is unfairly priced – as in the case of Radicava ORS – this results in higher barriers to access to the treatment for the ALS population, due to a potentially lengthy appeals process. Substantial delays in care such as those that may occur with appeals may harm patients, as patients may experience substantial progression in disease while waiting for treatment. This was highlighted by the experience of one person newly diagnosed with ALS. The patient's neurologist had recommended treatment with Radicava ORS. However, the patient faced repeated coverage denials for Radicava ORS based on the differing interpretations of the ALS Functional Rating Score Revised (ALSFRS-R); while the neurologist found this patient to be a good candidate for this treatment based on daily physical function, which the insurer and the pharmacy benefit manager chose to not consider, the patient's lower ALSFRS-R score was used by the insurer to deny coverage. After over 50 days disputing the denial of coverage, including an expedited Administrative Law Judge hearing, this patient ultimately gained approval for Radicava ORS based on the patient submitting video confirmation of the neurologist's assessment of physical function. As this example illustrates, insurance policies that use strict criteria based on clinical scales that may not fully reflect functional status (e.g., ALSFRS-R) can cause a substantial barrier to access and result in clinically relevant delays in care.

Access to Obesity Medications

The Obesity Action Coalition (OAC) submitted a number of different patient stories that illustrated important access challenges to obesity treatments. For some patients, their experience was related to numerous prior authorization hurdles and often ended with an ultimate coverage denial from their insurance provider. In one case, this denial was due to their employer not covering the obesity medication. Five other patient stories shared challenges with losing coverage to an obesity medication (Ozempic, Zepbound, Wegovy, Mounjaro) that was previously covered by their insurance (although Ozempic and Mounjaro are not indicated for obesity, ICER recognizes these treatments are often used off-label in this patient population). This withdrawal of coverage has been happening more frequently due to the high costs of obesity medications.[7] Half of these patients were given no warning about the change in coverage and only learned about it when they went to a pharmacy to refill their prescriptions. As a result, most patients report gaining back the weight they had successfully lost while having access to these medications.

The OAC also shared the results of their annual membership survey data that included questions and responses related to barriers to accessing obesity care. From 2020 – 2023, the biggest barriers to obesity care have been "services outright excluded" followed by "copay/deductibles too high" followed by "issues with a provider."[8] The majority of survey respondents would like "help in dealing with coverage denials/limitation" and "help finding what obesity treatments are available to me." Respondents had most experience with phentermine products (32%-40% from 2020-2023) while experience with Wegovy was up from 7% in 2021 to 22% by 2024. With regard to experience with methods of weight management, respondents had most experience with "self-directed behavior modification" followed by "health care provider-led behavior modification" rather than medications for obesity management.

Finally, we heard from OAC that Wegovy is excluded from 99% of Affordable Care Act marketplace policies, which is aligned with the biggest barrier reported in the membership survey above – "services outright excluded."[9] Compared to all the drugs in this year's report, Wegovy also had the lowest rate of concordance for cost sharing among the commercial policies that ICER reviewed.

Access to Gene Therapies for Beta Thalassemia and Hemophilia

We heard from the beta thalassemia community that since Zynteglo was only approved in August of 2023, very few patients to date have completed gene therapy; some are partially through the treatment process. Patients with commercial insurance were reported to not have difficulty accessing Zynteglo; most issues seemed to be related to Medicaid coverage. We also heard that barriers related to site of care and age are much greater issues for access for patients than coverage denials made through payer policies.

The hemophilia community has had a more "wait and see" approach to gene therapy. So far, uptake for Roctavian for Hemophilia A and Hemgenix for Hemophilia B has been limited to a small number of patients. Numerous factors may account for the slow uptake of hemophilia gene therapy:

- a community history of iatrogenic harm from (formerly-novel) therapies;
- patient concerns about the variability, durability, and potential side effects of first generation gene therapies (and patient recognition that recipients of existing gene therapy products will likely be ineligible for any second generation, Adeno-associated virus [AAV] based gene therapy);
- hesitancy about submitting to the requirements for receiving gene therapy (abstinence from alcohol, use of barrier contraception, administration of steroids); and
- a general unwillingness to switch from established first-line therapies that have been working "well enough" for some.

In addition to the concerns regarding gene therapy, some hemophilia patients may encounter barriers to access stemming from reimbursement issues with hospital administrators as well as limited sites of care. The complex administration of novel gene therapies has been appropriately limited to qualified hemophilia treatment centers; however, these centers are not always geographically accessible to patients. Barriers to access also exist among all hemophilia therapies (not just gene therapies) as hemophilia patients increasingly contend with burdensome prior authorization and step therapy criteria, and with formularies that subject all therapies to the highest level of cost-sharing tier, should they be covered at all.

As these survey 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 alone, and are meant to add additional dimensions to our collective understanding of "fair access" beyond the systematic assessment of published coverage policies.

6. Discussion

This assessment set out to evaluate whether coverage policies for drugs evaluated in ICER reviews during the calendar year 2022 were covered by major payers as of July 12, 2024 in concordance with fair access criteria for cost sharing, clinical eligibility, step therapy, and prescriber restrictions. Exploratory analyses were also performed to assess consumer access to drugs at the pharmacy, and on the degree of transparency of policies regarding clinical coverage criteria, cost sharing and tiering, and site of care for the three gene therapies in this year's scope. 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. This year, we attempted to address this aspect of access with our analysis of IQVIA consumer access data, and surveys and stories from patients and patient advocacy groups. Nevertheless, these limitations are important to keep in mind when considering the results of the assessment, which found a high level of concordance of coverage policies with fair access criteria across the top 10 largest payers and their largest formularies, as well as the Veterans Health Administration.

As with prior years, there was a high overall level of concordance across the fair access criteria. Non-concordance was clustered around the cost-sharing criteria, and mainly in one condition area, treatments for obesity. As noted earlier, despite obesity medications such as Wegovy and Qsymia being priced as cost-effective from a long-term value perspective, three payers each did not have Wegovy and Qsymia, or a therapeutic alternative, on the lowest relevant tier, which may result in decreased access. However, we acknowledge that the judgements made for this year's report that deemed those payers non-concordant do not fully reflect the complexities of obesity treatment. For example, we heard from payers who believed that coverage of the generic drug phentermine on the lowest relevant tier should meet fair access criteria. However, conversations with clinical experts suggested that long-term treatment with phentermine was not necessarily considered as a standard first-line therapy in most cases. As treatments for obesity continue to evolve, it will be important for clinical specialty societies to publish updated guidelines to help ensure payer coverage decisions are consistent with contemporary clinical practice.

Additionally, some drugs like Wegovy have now shown clinical benefits beyond obesity management (e.g., cardiovascular risk reduction, slowing of renal decline). This complicates payer decisions on coverage for several reasons. First, data are not yet available on whether obesity medications are superior to other already approved drugs that provide similar benefits (e.g., sodium-glucose cotransporter 2 inhibitors [SGLT-2i]). Also, although obesity medications may provide long-term value due to future cost-offsets from preventing complications of obesity, the upfront cost may affect payers' willingness to cover these drugs. For example, ICER estimated in its

2022 report that less than 1% of patients could be treated within 5 years without crossing the ICER potential budget impact threshold. As a result, payers are either declining to cover, stopping coverage, or putting restrictive clinical eligibility criteria for coverage.[10,7] Only around 40% of large employers cover GLP-1 drugs, and those that do cover the drugs usually require patients to meet prior authorization criteria.[11] Our exploratory analysis examining consumer access and stories from patients confirm that access to these drugs can be difficult and that out-of-pocket costs are high. Because drugs with higher cost sharing have been found to be associated with less adherence and higher discontinuation rates, consistent coverage of and placement of fairly-priced drugs such as Wegovy and Qsymia on preferred tiers may increase adherence and provides incentives for manufacturers to price drugs according to value at launch, thus decreasing costs for both payers and patients.[12]

In this year's report, we also evaluated three gene therapies. Overall, for the policies we evaluated, there was high concordance across the fair access criteria. However, the actual experiences of patients were much more variable. For some gene therapies, uptake and thus patient experience has been limited. For patients who have received or tried to obtain gene therapy, obstacles included limited sites of care offering treatment, age, and insurance barriers, particularly for those with Medicaid coverage. Additionally, our exploratory transparency analyses, which focused on the three gene therapies, found that it was difficult to find information on cost sharing, and site of care as a prospective plan member. There are an increasing number of gene therapies coming to market; for patients who qualify for and are potentially considering gene therapy, this is key information that may affect their choice of insurance plan and their access to care. To achieve greater transparency, payers should post clinical eligibility criteria for gene therapies in the patient-facing areas of their website.

While 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, our exploratory analysis of IQIVA data suggests that real-world access – the process between a prescription being written and a patient taking home the medication – may not necessarily be as straightforward as the high concordance rates suggest. The IQVIA data show a substantial number of prescriptions are rejected on the first attempt to fill the prescription, mainly due to non-coverage or utilization management strategies such as prior authorization or step therapy. Barriers to access existed regardless of whether a drug was priced below traditional cost-effectiveness thresholds – i.e., access to drugs considered cost-effective was not necessarily better than non-cost-effective drugs. Although utilization management is designed to encourage appropriate, evidence-based utilization of drugs and help manage resources across populations (ref Fair Access Criteria) – and in many cases it does – in practice, implementation of utilization management strategies may also pose additional barriers to access and burdens on the healthcare system. 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, and such measures may not only delay care but also may contribute to physician burnout.[13,14]

The cost-sharing data from IQVIA suggested that if a drug is covered by a payer, it is done so at relatively modest out-of-pocket costs, at least initially. However, the data only tracked new-to-brand prescriptions, and many manufacturers supply copay assistance for new prescriptions for a limited period of time. Additionally, we do not have information on potential copay adjustment programs (e.g., copay accumulators or maximizers) that may impact patient out-of-pocket costs, particularly in the long-term. Conversely, for prescriptions that were filled outside of insurance, patient out-of-pocket costs were high, since cash pay prescriptions are typically not eligible for copay assistance programs. For context, copay assistance is only available for members with a copay as a result of their benefit, while other patient assistance programs exist for cash pay members. Particularly for the obesity medications, which many plans exclude from coverage, these high costs may contribute to existing disparities in treatment.[15]

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. 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.[16] 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.[17,18] This year's report, which includes drugs to treat obesity and gene therapies, highlights the difficulties insurers and plan sponsors face when trying balance coverage and cost considerations.

As noted throughout this report, there are important limitations to our analysis which should color any conclusions. Perhaps foremost, we were unable to evaluate at the plan level 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. This year's exploratory analyses on consumer accessibility and out-of-pocket costs at the point-of-sale and transparency, as well as stories from patients, have given a glimpse of some of the challenges. However, without data that are tied to plan-specific policies, it is impossible to draw conclusions about specific barriers to access. Lack of transparency regarding deductibles also makes it difficult to draw conclusions about the true overall out-of-pocket costs incurred by individual

patients, particularly over a plan year. It is also possible that the 11 formularies selected for this assessment provide different coverage, which may be more or less consistent with fair access criteria than formularies with different tiering structures or from smaller payers, although prior reports that included smaller payers did not find substantial differences based on payer size. Finally, we were not able to capture issues outside of coverage policies (e.g., drug shortages, geographical barriers, etc.) that may affect access.[19]

7. Conclusion

This is ICER's fourth 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, our results continue to be a reflection on the limitations of the evidence available to us. The results of our assessment are useful in understanding how well written policies meet fair access criteria; however, 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. While for the first time we were able to access data from IQVIA on pharmacy fills, rejections, out-of-pocket costs, and cash pay prescriptions to help understand real-world access to the drugs in scope, these data were not tied to specific plans and thus we are not able to directly correlate them with our concordance analyses. Therefore, our ability to see through the complexity and opacity of these systems continues to remain limited. Furthermore, our analyses do not fully take into consideration larger contextual issues that may affect coverage, including the continued rising cost of drugs and the tradeoffs plan sponsors may make between ideal coverage and keeping costs at a reasonable level.

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 sometimes leads to positive change. The IQVIA prescription fill data shows that for many drugs, patient access at the point-of-care is uneven, particularly for obesity medications, which many plans do not cover. As a result, there are a substantial number of patients incurring high out-of-pocket costs.

Our reports continue to demonstrate that greater transparency is needed around coverage policies for new drugs, this year with a focus on gene therapies; this is becoming more urgent as additional gene therapies with very high up-front costs come to market. 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.

References

1. Claxton G, Rae, M, Winger, A, Wager, E, Byers, E, Kerns, J, Shmavonian, G, Damico, A. Employer Health Benefits 2024 Annual Survey 2024;2024. https://www.kff.org/report-section/ehbs-2024-section-9-prescription-drug-benefits/. Accessed October 10, 2024
2. Orenstein C. Are Copay Coupons Actually Making Drugs More Expensive? 2016. https://www.propublica.org/article/are-copay-coupons-actually-making-drugs-more-expensive. Published June 30, 2016. Accessed September 26, 2024.
3. Claxton G, Rae, M, Winger, A, Wager, E, Kerns, J, Shmavonian, G, Damico, A,. Employer Health Benefits 2023 Annual Survey 2023. https://www.kff.org/report-section/ehbs-2023-section-9-prescription-drug-benefits/. Accessed September 10, 2024
4. Anthem. Medical Policies & Clinical UM Guidelines Information for California https://www.anthem.com/ca/provider/policies/clinical-guidelines/. Published 2024. Accessed August 6, 2024.
5. The ALSA. ALS Focus Wave 4 – Telehealth Survey. In: The ALSA, ed. V1 ed: Harvard Dataverse; 2022.
6. ALS Association. ALS Focus Results from the Understanding Insurance Needs and Financial Burdens Survey. https://www.als.org/research/als-focus/survey-results/survey-1-results. Published 2020. Accessed September 10, 2024 2024.
7. Klein HE. Rising Costs Lead Insurers to Drop Weight Loss Drug Coverage, Further Increasing Patient Burden *American Journal of Managed Care.* 2024;30(Spec No. 10):SP781-SPP782 doi:https://doi.org/10.37765/ajmc.2024.89614
8. Obesity Action Coalition. Obesity Action Coalition, Annual (or Longitudinal) Membership Survey Data, 2023. Accessed with permissions: July 25, 2024.
9. Lo J, Cox, J. Insurer strategies to control costs associated with weight loss drugs 2024. https://www.healthsystemtracker.org/brief/insurer-strategies-to-control-costs-associated-with-weight-loss-drugs/#Share%20of%20ACA%20Marketplace%20plans%20including%20GLP-1%20agonists%20in%20formularies. Accessed September 26, 2024.
10. Atlas S, Kim, K, Beinfeld, M, Lancaster, V, Nhan, E, Lien, PW, Shah, K, Touchette, DR, Moradi, A, Rind, DM, Pearson, SD, Beaudoin, FL. Medications for Obesity Management: Effectiveness and Value; Final Evidence Report 2022. https://icer.org/assessment/obesity-management-2022/. Accessed December 22, 2023.
11. Chuang IZ, Welliver, S. Designing fiscally viable coverage of GLP-1 weight-loss drugs 2023. https://www.mercer.com/en-us/insights/us-health-news/designing-fiscally-viable-coverage-of-glp-1-weight-loss-drugs/. Published September 14, 2023 Accessed September 27, 2024.
12. Ismail WW, Witry, M. J., Urmie, J. M. The association between cost sharing, prior authorization, and specialty drug utilization: A systematic review. *J Manag Care Spec Pharm.* 2023;29(5):449-463. doi:10.18553/jmcp.2023.29.5.449. Accessed May 2024.
13. Howell S, Yin, P. T., Robinson, J. C. Quantifying The Economic Burden Of Drug Utilization Management On Payers, Manufacturers, Physicians, And Patients. *Health Aff (Millwood).* 2021;40(8):1206-1214.
14. American Medical Association. AMA survery indicates prior authorization wreaks havoc on patient care 2024. https://www.ama-assn.org/press-center/press-releases/ama-survey-indicates-prior-authorization-wreaks-havoc-patient-care. Accessed June 18, 2024.

15. Artiga S, Hill, L. What are the Implications of New Anti-Obesity Drugs for Racial Disparities? 2023. https://www.kff.org/policy-watch/what-are-implications-of-new-anti-obesity-drugs-racial-disparities/. Published July 24, 2023 Accessed September 27, 2024
16. Peter G. Peterson Foundation. Healthcare Costs for Americans Projected to Grow at an Alarmingly High Rate. Peter G. Peterson Foundation, . https://www.pgpf.org/blog/2019/05/healthcare-costs-for-americans-projected-to-grow-at-an-alarmingly-high-rate. Published 2019. Updated May 1, 2019. Accessed August 22, 2023.
17. Sherman M, Gregory, D, Parent, J, Wagner AK. Prescription Medications Account For One In Four Dollars Spent By A Commercial Health Plan. *Health Affairs Blog.* 2018. https://www.healthaffairs.org/content/forefront/prescription-medications-account-one-four-dollars-spent-commercial-health-plan. Accessed September 9, 2024.
18. Rome BN, Egilman, A.C., Kesselheim, A.S. Trends in Prescription Drug Launch Prices, 2008-2021. *JAMA.* 2022;327(21):2145-2147. doi:10.1001/jama.2022.5542.
19. Pearson SD; Lin G; Seidner M; Herce-Hagiwara B; Herron-Smith S; Lee M; Cummings M; Richardson M; Koola C; Emond SK. Assessment of Barriers to Fair Access 2023. https://icer.org/policy-papers/fair-access-coverage-policies-in-2023/.