Launch Price and Access Report

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No member of the Working Group, nor of any of the data partners, is responsible for the final contents of this report, nor should it be assumed they support any part of it. The report should be viewed as attributable to the ICER team.

About ICER

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List of Acronyms and Abbreviations Used in this Report

ACA	Affordable Care Act
ASP	average sales price
ATTR-CM	transthyretin cardiac amyloidosis
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
CI	confidence interval
CMS	Centers for Medicare and Medicaid
COPD	chronic obstructive pulmonary disease
ESI	employer-sponsored insurance
EUA	Emergency Use Authorization
evLY	equal value life year
FDA	The US Food and Drug Administration
FSS	federal supply schedule
GDP	gross domestic product
GLP-1 RAs	GLP-1 receptor agonists
HBPB	Health Benefit Price Benchmark
ICER	Institute for Clinical and Economic Review
LAAD	IQVIA's Longitudinal Access and Adjudication Data
MASH	metabolic dysfunction-associated steatohepatitis
MDS	myelodysplastic syndromes
MFN	most favored nation
MLD	metachromatic leukodystrophy
MRI	magnetic resonance imaging
N	number
NORD	National Organization for Rare Disorders
OOP	out of pocket
PAH	pulmonary arterial hypertension
PNH	paroxysmal nocturnal hemoglobinuria
QALY	quality-adjusted life year
SPEC	Tufts Medical Center Specialty Drug Evidence and Coverage database
UM	utilization management
US	United States
VIF	Variance Inflation Factors
WAC	Wholesale Acquisition Cost

Executive Summary

The launch prices of drugs in the United States (US) have been rising significantly over the past few decades, with many new medications entering the market at prices exceeding $200,000 annually.[1,2] This trend has sparked ongoing discussions about whether these high launch prices are justified and if they correspond to the clinical benefits provided to patients.[3-5] Further, many drug makers have produced data showing net prices on drugs are falling year-over-year, but those analyses do not provide drug-by-drug data and only include prices in aggregate across a manufacturer's entire portfolio, leaving policymakers to question the contribution of newly launched drugs to overall pricing trends.[6] A critical aspect of the launch price debate is the impact of launch prices on patient access to new therapies. Coverage delays or exclusions for newly launched drugs have become common. While policymakers, researchers, and other stakeholders have not always agreed on how to tackle these issues, improving drug affordability and patient access remains one of the few areas of bipartisan consensus in the US. To contribute to this ongoing policy discussion, we have developed a new annual report that evaluates the launch prices of new Food and Drug Administration (FDA)-approved drugs and patient access to these therapies. These new reports aim to assess the year-to-year trend in launch prices, evaluate the health system impact of not aligning launch prices with overall health benefits, and assess patient access barriers to newly launched drugs.

For this year's report, ICER (Institute for Clinical and Economic Review) analyzed launch price trends over a three-year period (2022-2024). In total, 154 novel agents approved by the US FDA Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) over the three-year period were included. ICER's analysis showed a 24% increase in the inflation-adjusted median annual launch price (list price) from 2022 to 2024. There was a larger 51% increase in the median annual net launch price (i.e., the actual amount the manufacturer receives after rebates, discounts, and other reductions) after adjusting for inflation. Given the differences in the mix of drugs approved each year, a multiple linear regression model was used to adjust for various drug characteristics that may impact the launch prices. Drug characteristics associated with significantly higher launch prices included gene or cell therapies, orphan products, and specific therapeutic areas, including oncology drugs and endocrine/metabolic drugs. After accounting for these drug characteristics, the annual launch price (list price) increased by 25% per year, while the annual net launch price increased by 33% per year, indicating that drug launch prices continue to rise at a rate that exceeds inflation, gross domestic product (GDP) growth, and overall health care cost growth.

Further assessments were conducted on 23 drugs that had been previously reviewed by ICER. The net prices of these drugs were compared with ICER's Health Benefit Price Benchmark (HBPB). ICER's HBPB suggests a price range, net of any discounts and rebates, that aligns fairly with the overall health benefits the treatment provides for patients over their lifetime, based on the data available

at the time of drug approval. Prices at or below these thresholds help ensure that the health benefits gained by patients using new treatments are not outweighed by health losses resulting from long-term cost pressures that lead individuals to become under-insured or uninsured. Table ES1 on the following page presents 16 of the 23 ICER-reviewed drugs (~70%) that had annual net prices exceeding ICER's upper-bound HBPB. For these 16 drugs, the estimated first-year drug spending was $1.92 billion, while expected spending at ICER's HBPB bounds would have been $431–661 million, resulting in first-year excess drug spending of $1.26–$1.49 billion.

To contextualize the consequences of this first-year excess drug, ICER estimated the health benefits foregone due to paying above the price benchmark (i.e., health opportunity costs). Based on previous established methodology in the literature, ICER estimated that the first-year excess drug spending of $1.26–1.49 billion would translate to 12,626–14,925 equal value life years (evLYs) lost – health benefits lost because the excess drug spending was not directed to other high-value interventions or services - assuming that each $100,000 spent is equivalent to an evLY lost. In other words, overspending on 16 of the 23 drugs reviewed in the first year post-launch resulted in more than 12,000 fewer life years in full health than could have been achieved with optimal spending across the US population. Assuming that the excess drug costs are fully passed on to all US enrollees as premium increases, ICER also estimated that the first-year excess spending could translate into 97,395–115,080 individuals losing insurance coverage. Based on data linking insurance loss to mortality, this estimated coverage loss would result in 351–415 deaths. Our scenario analysis showed that the number of individuals losing insurance coverage, along with the resulting deaths, was directly proportional to the share of excess drug costs that were passed through as premium increases. For example, if we assume that only 50% of the excess drug costs are transferred to US enrollees, the estimates for the number of individuals losing coverage and the associated deaths would effectively be reduced by half.

There are important limitations to these findings:

- Actual net pricing data are not transparent, and the estimates we used from the best available sources may differ from the true net price. To mitigate this uncertainty, we allowed drug manufacturers whose products were included in the excess drug spending analysis to correct their net price estimates; manufacturer-provided estimates superseded all other sources. Only two manufacturers provided net estimates.
- There is no formally established health opportunity cost in the US. Our assumption of $100,000 per evLY aligns with the cost-effectiveness thresholds commonly used in the US and with a simulation study estimating health opportunity costs in the US population.[7]
- The impact of excess drug spending on coverage loss and associated mortality depends on several uncertain parameters, such as the elasticity of insurance loss with respect to premium changes and the number needed to lose insurance to cause one death. Probabilistic sensitivity analysis is presented in the report to address parameter uncertainty.

Table ES1. ICER-Reviewed Drugs with Annual Net Prices Exceeding ICER's Upper-Bound HBPB

Drug Name	Annual Net Price (Best Estimate)	ICER HBPB†	Discount Needed to Meet HBPB‡
Approved in 2022			
Carvykti® (ciltacabtagene autoleucel)*	$465,000	$230,000 ~ $312,000	32.9% ~ 50.5%
Camzyos™ (mavacamten)	$64,127	$12,000 ~ $15,000	76.6% ~ 81.3%
Relyvrio® (sodium phenylbutyrate and taurursodiol)§	$130,401	$9,100 ~ $30,700	76.5% ~ 93%
Briumvi™ (ublituximab-xiiy)	$66,445	$16,500 ~ $34,900	47.5% ~ 75.2%
Approved in 2023			
Leqembi® (Lecanemab-irmb)	$26,491	$8,900 ~ $21,500	18.8% ~ 66.4%
Veozah™ (fezolinetant)	$3,998	$2,000 ~ $2,600	35% ~ 50%
Roctavian® (valoctocogene Roxaparvovec-rvox)*	$2,446,875	$1,960,000 ~ $1,960,000	19.9% ~ 19.9%
Fabhalta® (iptacopan)	$462,000	$178,000 ~ $180,000	61% ~ 61.5%
Casgevy® (exagamglogene autotemcel [exa-cel])*	$2,200,000	$1,350,000 ~ $2,050,000	6.8% ~ 38.6%
Lyfgenia® (lovotibeglogene autotemcel)*	$3,100,000	$1,350,000 ~ $2,050,000	33.9% ~ 56.5%
Approved in 2024			
Lenmeldy™ (atidarsagene autotemcel)*	$4,250,000	$2,294,000 ~ $3,940,000	7.3% ~ 46%
Attruby® (acoramidis)	$183,404	$13,600 ~ $39,000	78.7% ~ 92.6%
Winrevair™ (sotatercept-csrk)	$196,112	$17,900 ~ $35,400	81.9% ~ 90.9%
Voydeya™ (danicopan)	$38,390	$12,300 ~ $13,100	65.9% ~ 68%
Ohtuvayre™ (ensifentrine)	$35,400	$7,500 ~ $12,700	64.1% ~ 78.8%
Rytelo™ (imetelstat)	$352,115	$94,800 ~ $113,000	67.9% ~ 73.1%

HBPB: Health Benefit Price Benchmark, ICER: Institute for Clinical and Economic Review

*One-time administered gene or cell therapies

†ICER's HBPB is presented as a range

‡Discount from net price required to reach ICER HBPB for drugs priced above its upper bound

§We acknowledge that Relyvrio was withdrawn from the market in 2024 based on negative Phase III results. As our focus was on price at launch, we included this drug in our analysis because it was on the market for over a year.

To evaluate the patient access barriers to newly launched drugs, ICER focused on the novel drugs approved in 2024. We identified data on 24 drugs from the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) database, which contains information on specialty drug coverage decisions issued by up to 18 large US commercial health plans. For the majority of these drugs, insurance coverage policies were lacking, even up to one year after approval, which may reflect the impact of new-to-market blocks. We also obtained first-quarter 2025 information from IQVIA's Longitudinal Access and Adjudication Data on 17 drugs with at least 100 total commercial written prescriptions that quarter. The data showed that the majority of commercial new-to-brand (i.e., first-time) prescriptions for newly approved drugs were rejected. On average, only 29% of total

dispensed commercial new-to-brand prescriptions were ultimately successfully filled overall. Rejection rates were greater than 50% regardless of whether the drug was first-in-class, considered an orphan drug, or deemed cost-effective by ICER. Non-coverage of the drug was the most common reason for rejection, perhaps reflecting that the establishment of coverage policies often significantly lags approval dates. Patient out-of-pocket costs varied, likely due to the variation in cost-sharing policies that are set by plan sponsors. Overall, a large proportion of new-to-brand prescriptions had a $0 out-of-pocket cost, likely due to multiple reasons, including the use of manufacturer assistance programs (e.g., "copay cards"), which patients can use to obtain their first few prescriptions at little to no cost to them. Finally, ICER conducted facilitated group discussions with patient advocacy groups to discuss access challenges. Patient advocates described other important factors, beyond insurance coverage, that impact access, including health system complexity, health inequities, drug burden, and other cost-related issues.

This report, even with its important limitations, contributes to the critical decisions facing the US health care system. Health insurance premiums are rising at unsustainable rates, and significant increases in the number of uninsured are expected. By evaluating the net price trends of newly-launched pharmaceutical products and identifying opportunities to enhance affordability by adopting ICER's HBPB, we aim to highlight value-based pricing as an important approach to determining fair pricing that rewards innovation without causing harmful effects on patients through higher health insurance premiums. While there are often other practical considerations, such as the eligible patient population size, that may be relevant to a drug's pricing, these decisions are too often made without rigorous evidence and with little transparency. More communication from drugmakers on how prices are determined, along with greater transparency on net prices, will help policymakers address affordability in a more systematic and evidence-based manner.

Additionally, although our evaluation of patient access was limited, our results highlight various barriers to patient access, including insurance- and non-insurance-related barriers. The gaps in coverage availability particularly highlight the impact of the new-to-market block on insurance coverage. Although, in principle, these blocks can be justified to allow an insurer adequate time to review the clinical evidence, the lack of timely coverage policies could limit or delay access, as without a policy, patients would need to engage in lengthy exception or prior authorization processes. Thus, we emphasize the need for early evidence evaluation by payers and the importance of early data sharing by drug makers to enhance patient access.

1. Introduction

1.1. Background

The launch prices of drugs in the United States (US) have been rising significantly over the past few decades, with many new medications entering the market at prices exceeding $200,000 annually.[1,2] This trend has sparked ongoing discussions about whether these high launch prices are justified and if they correspond to the clinical benefits provided to patients.[3-5] In addition, accelerating growth in prescription drug spending is contributing to an increase in overall health spending,[8] and significantly contributing to premium increases.[9] One analysis of net launch prices of cancer drugs from 2008 to 2022 did not find that higher prices were associated with better clinical efficacy.[10] Manufacturers often cite the substantial costs of innovation as a reason for these prices; however, studies have shown no significant correlation between how much a company spends on research and development and the price of the drugs.[11,12] Complicating these discussions is the fact that the net price of a drug – i.e., the actual amount the manufacturer receives after rebates, discounts, and other reductions – often differs from the list price. However, determining the overall net price can be complex, as net prices are proprietary and vary significantly among different payers due to market conditions and statutory requirements. Additionally, the impact of government regulations—such as the Medicare drug price negotiation provision of the Inflation Reduction Act—on launch prices remains unclear. Some predict that Medicare drug price negotiation could lead to further increases in launch prices as the industry responds to potential price reductions in the future.[13,14]

Another critical aspect of the launch price debate is the impact of launch prices on patient access to new therapies. Coverage delays or exclusions for newly launched drugs have become common.[15] For example, over half of new prescriptions for novel medications go unfilled, often due to lack of insurance coverage.[16] Even when these drugs are covered, utilization management strategies (e.g., prior authorization, step therapy) can create barriers to access and delay care – in 2024, Medicare Part D and Medicare Advantage plans employed utilization management strategies for more than half of the drugs listed on their formularies.[17] Additionally, high co-pays and deductibles can create financial burdens for patients and barriers to adherence and persistence. An online survey of nearly 3,000 US adults with chronic health conditions conducted in 2025 found that prescription medication access and affordability have declined over the past year.[18] Almost half of the respondents reported difficulties accessing prescription medications through their health plans, mainly due to coverage issues (18%), high out-of-pocket costs (18%), prior authorization (16%), and high deductibles (13%). In addition, over 20% of patients reported difficulties paying for prescriptions, and approximately the same proportion indicated they could not obtain necessary prescriptions due to cost, putting their health at risk. The ongoing tension between the high costs of treatments and the standard methods payers use to manage these costs may hinder patients from

receiving appropriate, evidence-based, and patient-centered care. Many studies have indicated that although the US spends more on health care and prescription drugs than other high-income countries, Americans experience worse health outcomes and access to care.[5,19]

Improving drug affordability and patient access remains one of the few areas of bipartisan consensus in the US; however, policymakers, researchers, and other stakeholders have not always agreed on how to tackle these issues. To contribute to this ongoing policy discussion, the Institute for Clinical and Economic Review (ICER) publishes this annual report to evaluate the launch prices of new Food and Drug Administration (FDA)-approved drugs and patient access to those therapies.

2. Scope of Work and Approach

In January 2025, we organized a multi-stakeholder working group to provide input into the approach and methods for this report. The working group was comprised of representatives from patient and consumer advocacy groups, pharmaceutical companies, clinical experts, purchasers, and insurers. Working with this group, we developed a Launch Price and Access Protocol that was previously published. Below we present a summary of our approach; see Appendix A and B for detailed methodology.

2.1. Launch Price Evaluation

Given the interest in understanding changes in launch prices over the years, our scope included all novel agents approved by the US FDA Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) over three years (January 2022 to December 2024). Novel agents are new drugs that have never been approved or marketed in the US, and therefore do not include generics, biosimilars, or drugs that have been previously approved for other indications. From the list of novel agents, we excluded vaccines, antibiotics, microbiota products, blood or plasma-based products, and imaging or diagnostic agents due to different pricing strategies and market dynamics. See Table 2.1.

For the launch price evaluation, we conducted two broad analyses:

1. Trend Analyses: We analyzed launch price trends over three years (2022 to 2024), as well as the impact of various relevant drug characteristics, such as drug type, therapeutic area, and population size, on list and net prices (i.e., the actual amount the manufacturer receives after rebates, discounts, and other reductions).

2. Health System Impact Analyses: We conducted an in-depth review of the drugs in scope that have been previously reviewed by ICER. For these sets of drugs, we assessed their launch prices in relation to ICER's Health Benefit Price Benchmark (HBPB). ICER's HBPB suggests a price range, net of any discounts and rebates, that aligns fairly with the overall health benefits the treatment provides for patients over their lifetime, based on the data available at the time of drug approval. Prices at or below these thresholds help ensure that the health benefits gained by patients using new treatments are not outweighed by health losses due to long-term cost pressures that lead individuals to delay care, abandon care, or lose health insurance. For drugs that were priced above ICER's HBPB, we estimated potential savings in health care spending if priced within ICER's HBPB, and calculated opportunity costs associated with excess spending on these drugs.

Table 2.1. Scope: Launch Price Evaluation

	FDA Approval Year			Total Number of Drugs
	2022	2023	2024	
Number of Novel Drugs Approved*	40	60	54	**154**
Number Previously Reviewed by ICER	10	7	8	**25**

FDA: The US Food and Drug Administration, ICER: Institute for Clinical and Economic Review
*Excludes vaccines, antibiotics, microbiota products, blood or plasma-based products, imaging or diagnostic agents

Determination of List and Net Prices

Data on the manufacturer's list price (Wholesale Acquisition Cost [WAC]) at launch was obtained from Redbook. We converted unit WAC prices to annual WAC prices using the dosage information available on the FDA label. For drugs that required assumptions or placeholders to determine annual WAC estimates, we have outlined the inputs we used in Table A1.2 of the Appendix. Given the complexities and lack of transparency around net prices, we relied on multiple data sources to generate the best net price estimates for our analyses (see Figure A1.1 in Appendix). For provider-administered drugs, we prioritized Average Sales Price (ASP) data when available through the Centers for Medicare & Medicaid Services (CMS) ASP files, and removed the 6% markup that is included in the CMS payment allowance.[20] We prioritized ASP as a net price estimate for provider-administered drugs because it is manufacturer-reported and based on sales to most purchasers. However, we acknowledge some key limitations to using ASP as a net price estimate because these prices do not include discounted sales prices to certain entities, such as 340B-covered entities, state pharmaceutical assistance programs, and other federal programs.[21]

When ASP was not available for a provider-administered drug in scope, we used WAC as a placeholder. This is in accordance with the CMS policy for drug reimbursement, which states that drugs will be reimbursed at a rate of WAC with a 3% markup in the time between launch and before an ASP price is available. We did not include the 3% markup in our calculations. This approach was further supported by our analysis, which found that among the 41 provider-administered drugs with an ASP available, the first available ASP price was generally similar to the WAC price at launch. The median percent difference between WAC and ASP was 0.2295%, and the mean was 3.7%. ASP was within 1% of WAC at launch for more than half of these provider-administered drugs in our scope (22 of 41 drugs).

For non-provider-administered drugs, we used gross-to-net discount rates from multiple sources (Rebate Benchmark and Estimated Total Discounts from the IPD Analytics Rebate Monitor, forecasted discounts from the IPD Analytics Market & Financial Insights, and gross-to-net discount rates from SSR Health) to calculate net price. Detailed information about these sources is available in the Appendix. When more than one of these sources had data for a drug in scope, we calculated

the median of all available discount data. If data were not available from any of these sources, we used the federal supply schedule (FSS) price closest to the time of launch. If FSS data were also not available, we estimated the gross-to-net discount rate from the median discount among all other drugs in scope that were not provider-administered, which was 20%.

For drugs in our scope that had previously been reviewed by ICER, we sent our best net price estimates and other data inputs to manufacturers as part of our data validation process. When provided, manufacturer data submissions of net prices were used for the analyses, superseding all other data sources. Sensitivity analyses were conducted to account for uncertainties in the net price estimates and other data inputs.

2.2. Patient Access Evaluation

To evaluate patient access to newly approved drugs, we focused our analysis on the 54 novel drugs in scope that were approved in 2024 (see Table 2.2). Given the high demand for GLP-1 receptor agonists (GLP-1s) treatments that raise concerns about access and affordability, we also chose to include Zepbound® (tirzepatide), the most recently launched GLP-1 for obesity, as a drug of special interest, even though it was approved in 2023. Thus, there were a total of 55 drugs in scope.

Table 2.2. Scope: Patient Access Evaluation

	2024 Novel Drugs Approval*	Additional Drug of Special Interest†	Total Number of Drugs
Drugs in Scope	54	1	55

*Excludes vaccines, antibiotics, microbiota products, blood or plasma-based products, imaging or diagnostic agents
†tirzepatide (Zepbound)

To assess various aspects of patient access barriers, we obtained data from two primary sources: 1) the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) database, which contains coverage policies from 18 commercial payers, and 2) IQVIA's Longitudinal Access and Adjudication Data (LAAD), which contains real-world prescription fill data based on pharmacy transactions for those with commercial insurance and those paying cash. Utilizing information from these databases, we aimed to evaluate key aspects of access: coverage policy availability and restrictions, the burdens of prior authorization, and patient cost-sharing.

a) Coverage Policy Availability and Restrictions: We evaluated the availability of coverage policies and whether the drug is covered or not, and attempted to evaluate whether policy restrictions were consistent with ICER's Cornerstones of "Fair" Drug Coverage criteria.

b) Prior Authorization Burden: We examined the proportion of new-to-brand prescriptions that were filled, not filled (i.e., rejected), and abandoned for the commercial line of business. We further evaluated the reasons for rejection, including prior authorization and step therapy. We also examined whether certain drug characteristics, such as therapeutic area, first-in-class, or orphan drug status, showed any correlation with coverage.
c) Patient Cost-Sharing: We evaluated final out-of-pocket costs, both for patients with commercial insurance coverage (e.g., copay or co-insurance) and for patients who did not have or did not use insurance benefits (e.g., cash pay for prescription). We did not have data on prescriptions that were covered by non-commercial payers (e.g., Medicare, Medicaid).

Patient Voices on Access Challenges

To help add context to the data on patient access, we conducted facilitated group discussions with patient groups to discuss access challenges for eight drugs that ICER reviewed that were approved in 2024, in partnership with the National Health Council. Representatives from patient groups representing the eight drugs were invited to participate. Two group discussions were held, one focused on drugs for more common diseases and the other focused on drugs for rare diseases. The facilitated group discussions explored themes such as prior authorization, coverage restriction, patient assistance programs, and costs, including non-drug costs.

3. Launch Prices

Table 3.1 summarizes the launch prices, including the list price, and the best net price estimate for each drug in scope. List and net price data were not available for four drugs: Omlonti® (omidenepag isopropyl), Exxua™ (gepirone), Exblifep® (cefepime, enmetazobactam), and Unloxcyt™ (cosibelimab-ipdl). We excluded Paxlovid™ (nirmatrelvir, ritonavir) because the drug was used before its FDA approval under an Emergency Use Authorization (EUA) at prices negotiated by the US government.

Table 3.1. Launch Prices: Annual List and Net Prices

Drug Name [Brand Name (generic name)]	Annual List Price	Best Annual Net Price Estimate
2022 Approvals		
Skysona® (elivaldogene autotemcel)	$3,000,000.00	$3,000,000.00 †
Hemgenix® (etranacogene dezaparvovec-drlb)	$3,500,000.00	$2,646,633.00 ††
Zynteglo® (betibeglogene autotemcel)	$2,800,000.00	$2,138,693.47 ¤
Kimmtrak® (tebentafusp-tebn)	$1,294,440.00	$1,294,440.00 *
Xenpozyme™ (olipudase alfa-rpcp)	$871,324.00	$871,324.00 *
Carvykti® (ciltacabtagene autoleucel)	$465,000.00	$465,000.00 *
Amvuttra™ (vutrisiran)	$463,500.00	$459,986.23 *
Elahere™ (mirvetuximab soravtansine-gynx)	$431,253.25	$430,402.94 *
Tecvayli™ (teclistamab-cqyv)	$369,930.00	$365,966.89 *
Opdualag™ (nivolumab and relatlimab-rmbw)	$356,049.00	$354,467.32 *
Lunsumio™ (mosunetuzumab-axgb)	$340,395.98	$340,396.06 *
Enjaymo™ (sutimlimab-jome)	$340,200.08	$333,522.59 *
Rezlidhia™ (olutasidenib)	$391,766.69	$301,268.59 §
Pyrukynd® (mitapivat)	$335,800.00	$285,933.70 ‡
Pluvicto™ (lutetium Lu 177 vipivotide tetraxetan)	$255,000.00	$255,000.00 †
Adstiladrin® (nadofaragene firadenovec)	$240,000.00	$239,643.04 **
Lytgobi® (futibatinib)	$304,253.60	$232,394.46 ¤
Ztalmy® (ganaxolone)	$289,677.87	$231,742.30 #
Vonjo™ (pacritinib)	$237,250.00	$201,662.50 #
Krazati™ (adagrasib)	$240,291.62	$173,490.55 ‡
Tzield™ (teplizumab-mzwv)	$193,900.00	$163,430.19 *
Relyvrio® (sodium phenylbutyrate and taurursodiol)	$163,001.70	$130,401.36 ‡‡
Briumvi™ (ublituximab-xiiy)	$98,333.30	$66,445.19 *
Camzyos™ (mavacamten)	$89,499.93	$64,126.70 ‡
Terlivaz® (terlipressin)	$60,800.00	$60,800.00 †
Rolvedon™ (eflapegrastim-xnst)	$58,500.00	$51,433.05 *
Spevigo® (spesolimab-sbzo)	$51,133.00	$51,132.74 *
Imjudo® (tremelimumab-actl)	$48,750.00	$48,311.32 *
Sunlenca® (lenacapavir)	$43,062.50	$43,062.50 †

Drug Name [Brand Name (generic name)]	Annual List Price	Best Annual Net Price Estimate
Sotyktu™ (deucravacitinib)	$74,999.94	$40,781.22 ‡§§
Cibinqo™ (abrocitinib)	$59,787.00	$33,719.87 ‡
Vabysmo™ (faricimab-svoa)	$11,680.00	$10,323.69 *
Mounjaro™ (tirzepatide)	$12,666.29	$4,582.03 ‡
Vtama® (tapinarof)	$16,120.81	$4,497.71 ‡
Nexobrid® (anacaulase-bcdb)	$3,150.00	$3,150.00 †
Quviviq® (daridorexant)	$5,560.05	$2,085.02 ‡
Vivjoa™ (osteconazole)	$2,700.00	$2,021.07 ¤
Daxxify™ (daxibotulinumtoxinA-lanm)	$840.00	$564.53 *
Voquezna Triple Pak™ (vonoprazan, amoxicillin, clarithromycin)	$812.00	$430.36 #
2023 Approvals		
Elevidys® (delandistrogene Moxeparvovec-rokl)	$3,200,000.00	$3,200,000.00 †
Lyfgenia® (lovotibeglogene autotemcel)	$3,100,000.00	$3,100,000.00 †
Roctavian® (valoctocogene Roxaparvovec-rvox)	$2,446,875.00	$2,446,875.00 †
Casgevy® (exagamglogene autotemcel (exa-cel))	$2,200,000.00	$2,200,000.00 †
Lamzede® (velmanase alfa-tycv)	$1,456,000.00	$1,513,718.04 *
Vyjuvek® (beremagene geperpavec-svdt)	$1,261,000.00	$1,235,780.00 *
Veopoz™ (pozelimab-bbfg)	$1,168,269.00	$1,168,269.00 †
Rivfloza™ (nedosiran)	$754,560.00	$743,184.72 ¤
Pombiliti™ (cipaglucosidase alfa-atga)	$618,799.85	$605,345.58 *
Sohonos™ (palovarotene)	$624,150.00	$543,010.50 #
Joenja® (leniolisib)	$547,500.00	$533,853.75 ¤
Daybue™ (trofinetide)	$616,120.00	$502,445.86 ‡
Elrexfio™ (elrnatamab-bcmm)	$490,432.32	$490,432.32 †
Fabhalta® (iptacopan)	$550,000.03	$462,000.03 #
Zilbrysq® (zilucoplan)	$524,840.40	$440,865.94 #
Elfabrio® (pegunigalsidase alfa-iwxj)	$430,051.44	$438,658.26 *
Filsuvez® (birch triterpenes)	$583,200.17	$433,278.72 ¤
Wainua™ (eplontersen)	$498,999.94	$417,912.45 ‡§§
Vanflyta® (quizartinib)	$398,580.00	$398,580.00 †
Epkinly® (epcoritamab-bysp)	$396,170.11	$396,169.23 *
Talvey™ (talquetamab-tgvs)	$371,121.00	$366,151.91 *
Columvi™ (glofitamab-gxbm)	$349,999.38	$349,986.93 *
Omisirge® (omidubicel-onlv)	$338,000.00	$338,000.00 †
Augtyro™ (repotrectinib)	$363,412.25	$308,900.41 #
Lantidra™ (donislecel-jujn)	$300,000.00	$300,000.00 †
Ojjaara® (momelotinib)	$327,283.35	$294,555.01 #
Rystiggo® (rozanolixizumab-noli)	$290,400.00	$289,733.43 *
Skyclarys™ (omaveloxolone)	$375,138.90	$288,856.95 #
Ogsiveo™ (nirogacestat)	$352,837.69	$282,270.15 #

Drug Name [Brand Name (generic name)]	Annual List Price	Best Annual Net Price Estimate
Orserdu™ (elacestrant)	$259,880.00	$233,892.00 #
Qalsody™ (tofersen)	$213,450.01	$214,517.25 *
Fruzaqla™ (fruquintinib)	$327,600.00	$212,940.00 #
Jaypirca™ (pirtobutinib)	$255,500.00	$190,475.25 ‡
Zynyz™ (retifanlimab-dlwr)	$185,120.00	$182,944.34 *
Truqap™ (capivasertib)	$297,986.04	$178,791.62 #
Loqtorzi® (toripalimab-tpzi)	$154,128.52	$154,124.07 *
Agamree® (vamorolone)	$156,037.50	$132,631.88 #
Omvoh™ (mirkizumab-mrkz)	$150,229.00	$95,051.60 *
Filspari™ (sparsentan)	$120,450.00	$93,951.00 #
Ngenla™ (somatrogon-ghla)	$99,699.60	$85,165.00 ¤
Bimzelx® (bimekizumab-bkzx)	$66,387.60	$66,387.60 †
Velsipity™ (etrasimod)	$74,999.94	$55,687.46 ‡§§
Izervay™ (avacincaptad pegol)	$50,400.00	$50,400.00 *
Defencath® (taurolidine, heparin)	$38,998.44	$38,998.44 †
Litfulo™ (ritlecitinib)	$49,134.62	$33,534.38 ‡§§
Leqembi® (Lecanemab-irmb)	$26,500.24	$26,490.57 *
Ryzneuta® (efbemalenograstim alfa-vuxw)	$18,400.00	$18,400.00 †
Aphexda™ (motixafortide)	$11,800.00	$11,800.12 *
Zurzuvae™ (zuranolone)	$15,900.00	$10,812.00 #
Jesduvroq® (daprodustat)	$11,417.20	$8,375.29 ¤
Zavzpret™ (zavagepant)	$17,600.00	$6,160.00 #
Rezzayo™ (rezafungin)	$5,850.00	$5,729.43 *
Veozah™ (fezolinetant)	$6,691.65	$3,998.26 ‡
Inpefa™ (sotagliflozin)	$7,275.65	$3,161.27 ‡
Miebo™ (perfluorhexyloctane)	$9,252.00	$2,613.69 ‡§§
Xdemvy™ (lotilaner)	$1,850.00	$980.50 #
Beyfortus™ (nirsevimab-alip)	$495.00	$495.00 †
Brenzavvy™ (bexagliflozin)	$474.50	$379.60 ‡‡
2024 Approvals		
Lenmeldy™ (atidarsagene autotemcel)	$4,250,000.00	$4,250,000.00 †
Kebilidi™ (eladocagene exuparvovec-tneq)	$3,950,000.00	$3,950,000.00 †
Beqvez™ (elaparvovec-dzkt)	$3,500,000.00	$3,500,000.00 †
Ryoncil® (remestemcel-L-rknd)	$1,552,000.00	$1,552,000.00 †
Miplyffa™ (arimoclomol)	$967,432.50	$919,060.88 #
Alhemo® (concizumab-mtci)	$888,552.00	$755,269.20 #
Revuforj® (revumenib)	$810,984.37	$729,885.93 #
Tecelra® (afamitresgene autoleucel)	$727,000.00	$727,000.00 *
Hympavzi™ (marstacimab-hncq)	$795,600.00	$676,260.00 #
Bizengri® (zenocutuzumab-zbco)	$617,500.00	$617,500.00 †
Duvyzat® (givinostat)	$675,032.21	$573,777.38 #

Drug Name [Brand Name (generic name)]	Annual List Price	Best Annual Net Price Estimate
Tryngolza™ (olezarsen)	$595,008.00	$565,257.60 #
Aqneursa™ (levacetylleucine)	$701,321.42	$561,057.14 #
Ziihera® (zanidatamab-hrii)	$554,580.00	$553,652.83 *
Piasky® (crovalimab-akkz)	$551,839.55	$551,839.55 †
Anktiva® (nogapendekin alfa inbakicept-pmln)	$537,000.00	$537,000.00 *
Aucatzyl® (obecabtagene autoleucel)	$525,000.00	$525,000.00 †
Amtagvi® (lifileucel)	$515,000.00	$515,000.00 †
Crenessity™ (crinecerfont)	$466,384.83	$464,041.05 ¤
Xolremdi™ (mavorixafor)	$496,400.00	$446,760.00 #
Voranigo® (vorasidenib)	$485,218.83	$412,436.01 #
Imdelltra™ (tarlatamab-dlle)	$400,071.00	$395,210.40 *
Rytelo™ (imetelstat)	$354,780.69	$352,114.71 *
Niktimvo™ (axatilimab-csfr)	$319,410.00	$319,410.00 †
Ojemda™ (tovorafenib)	$330,720.00	$297,648.00 #
Alyftrek® (vanzacaftor, tezacaftor, and deutivacaftor)	$370,269.29	$296,215.43 #
Itovebi™ (inavolisib)	$298,087.68	$253,374.53 #
Yorvipath® (palopegteriparatide)	$285,808.04	$242,936.83 #
Ensacove™ (ensartinib)	$255,014.55	$204,011.64 #
Winrevair™ (sotatercept-csrk)	$245,140.00	$196,112.00 #
Lazcluze™ (lazertinib)	$221,409.00	$188,197.65 #
Tevimbra® (tislelizumab-jsgr)	$180,405.33	$187,099.61 *
Attruby® (acoramidis)	$244,538.52	$183,403.89 #
Vyloy® (zolbetuximab-clzb)	$175,968.00	$174,084.85 *
Livdelzi® (seladelpar)	$153,373.00	$138,035.70 #
Iqirvo® (elafibranor)	$139,430.00	$125,487.00 #
Leqselvi™ (deuruxolitinib)	$55,525.14	$44,420.11 ‡‡
Rezdiffra™ (resmetirom)	$48,058.35	$43,372.66 ‡§§
Ebglyss™ (lebrikizumab-lbkz)	$66,500.00	$39,900.00 #
Voydeya™ (danicopan)	$50,260.50	$38,389.97 ¤
Nemluvio® (nemolizumab-ilto)	$55,120.00	$35,828.00 #
Ohtuvayre™ (ensifentrine)	$35,400.01	$35,400.23 *
Kisunla™ (donanemab-azbt)	$31,999.90	$31,091.23 *
Symvess™ (acellular tissue engineered vessel-tyod)	$29,500.00	$29,500.00 †
Cobenfy™ (xanomeline and trospium chloride)	$22,508.31	$18,006.65 #
Vafseo® (vadadustat)	$15,549.00	$14,843.33 ¤
Rapiblyk™ (landiolol)	$12,950.00	$12,950.00 †
Sofdra™ (sofpironium)	$8,784.51	$6,149.16 #
Tryvio™ (aprocitentan)	$9,429.17	$5,563.21 #
Orlynvah™ (sulopenem etzadroxil, probenecid)	$2,975.00	$2,677.50 #
Zelsuvmi™ (berdazimer)	$1,950.00	$1,560.00 ‡‡
Letybo® (letibotulinumtoxinA-wlbg)	$660.00	$660.00 †

Net price sources: *ASP (Note that ASP is occasionally higher than WAC);[22] †WAC; ‡Median of multiple sources; §SSR Health; #IPD Analytics; ¤FSS; **Manufacturer data submission based on ASP; ††Manufacturer data submission based on FSS pricing; ‡‡Placeholder discount (20%). §§IPD Analytics uses the value of Non-Supply Chain Discount as a placeholder for drugs that do not yet have Rebate Benchmark data available.
Note: Four drugs were excluded from Table 3.1 because they did not have list and net prices available, despite being approved by the FDA between 2022-2024. One drug was excluded because it was launched prior to 2022 under the EUA.

3.1. Results of Analysis

Trend Analysis

We conducted analyses examining trends in launch prices (list and net prices) over a three-year period (2022-2024), independent of various drug characteristics. We reviewed year-on-year changes, as well as the total change over the three years. A total of 149 drugs were included in our trend analysis.

List and Net Price Changes

Because the data had a heavy skew, due to some extremely expensive launch prices, we report on the median list and net price instead of the mean. The median annual list and net prices are presented in Table 3.2. To account for inflation, the list and net price for drugs approved in 2022 and 2023 were adjusted to 2024 values.[23]

Overall, there was a 24% increase ($59,492) in the inflation-adjusted median annual list price of newly launched drugs from 2022 to 2024. Specifically, from 2022 to 2023, the median annual list price increased by 23% from $249,257 to $306,937. However, from 2023 to 2024, there was a much smaller 1% increase in median annual list price from $306,937 to $308,749 (Figure 3.1, Table 3.2).

Similarly, the inflation-adjusted median annual net price of newly launched drugs increased by 51% ($92,524) from 2022 to 2024. From 2022 to 2023, there was an increase of 45% from $182,271 to $264,938, while from 2023 to 2024 it increased by 4% from $264,938 to $274,795 (Figure 3.1, Table 3.2).

Table 3.2. Launch Price: Median Annual List and Net Price*

	Annual List Price		Annual Net Price	
Year	**Median (Range)**	**Median (Range) Adjusted to 2024***	**Median (Range)**	**Median (Range) Adjusted to 2024***
2022, n=39	$237,250 ($812-$3,500,000)	$249,257 ($853-$3,677,128)	$173,491 ($430-$3,000,000)	$182,271 ($452-$3,151,824)
2023, n=58	$298,993 ($474-$3,200,000)	$306,937 ($487-$3,285,018)	$258,081 ($380-$3,200,000)	$264,938 ($390-$3,285,018)
2024, n=52	$308,749 ($660-$4,250,000)	$308,749 ($660-$4,250,000)	$274,795 ($660-$4,250,000)	$274,795 ($660-$4,250,000)

n: number of drugs

*List and net price for drugs approved in 2022 and 2023 were adjusted for inflation to 2024 values.[23]

Figure 3.1. Annual Median List and Net Price with Year-on-Year Change Denoted by Vertical Arrows and Total Change Over Three Years Denoted by Horizontal Arrow

Adjusted List and Net Price Changes

Given the differences in the mix of drugs approved each year (Table A1.1), we fitted a multiple linear regression model to adjust for various drug characteristics that may impact the launch prices. Regression models provide estimations of which drug characteristics have the highest correlation with the outcome (i.e., list or net price).

Drug characteristics associated with significantly higher launch prices included gene or cell therapies, orphan products, and specific therapeutic areas, including oncology drugs and endocrine/metabolic drugs. We accounted for drug characteristics in our regression model to examine the underlying trend in launch price. This means that we evaluated the impact of year on list and net price while holding all the drug characteristics equal. After accounting for drug characteristics in the model, the annual list price still increased by 25% per year (95% CI: -6% to 67%), while the annual net price increased by 33% per year (95% CI: -0.5% to 79%). See Table 3.3.

We conducted quantile regression analyses estimating the median list and net price. The results of these analyses were consistent with the findings of the multiple linear regression. See Appendix Tables A1.11-A1.12.

Table 3.3. Adjusted Change in List and Net Price

	List Price Change	Net Price Change
2022 to 2024, n=149	+25% per year (95% CI: -6% to 67%; p=0.12)	+33% per year (95% CI: -0.5% to 79%; p=0.05)

n: number of drugs

Trends Across Drug Characteristics

In Figures 3.2 to 3.7 below, we present the trends in launch prices (list and net) of gene and cell therapies, orphan products, biologics, and small molecules over the three-year period. Additionally, we highlight the trends for two therapeutic areas that were significantly associated with higher launch prices: oncology and endocrinology. We advise caution when interpreting the launch price trends for gene and cell therapies, as well as endocrinology drugs, due to the limited number of drugs in these categories. Given that the distribution of characteristics and launch price trends in different subgroups varies across years, the adjusted rates presented above more accurately reflect the underlying trend in launch prices.

Figure 3.2. Median List and Net Price for Gene/Cell Therapies

Gene/Cell Therapies		
Year	**List Price**	**Net Price**
2022 (n=5)	$2,941,702	$2,246,929
2023 (n=6)	$2,385,166	$2,385,167
2024 (n=6)	$2,113,500	$2,113,500

n: number

Figure 3.3. Median List and Net Price for Orphan Products

Orphan Products		
Year	**List Price**	**Net Price**
2022 (n=24)	$357,520	$333,458
2023 (n=34)	$397,138	$392,525
2024 (n=33)	$515,000	$515,000

n: number

Figure 3.4. Median List and Net Price for Biologic Drugs

Biologic Drugs		
Year	**List Price**	**Net Price**
2022 (n=20)	$357,520	$354,013
2023 (n=24)	$370,140	$367,583
2024 (n=23)	$525,000	$525,000

n: number

Figure 3.5. Median List and Net Price for Small Molecule Drugs

Small Molecule Drugs		
Year	**List Price**	**Net Price**
2022 (n=16)	$132,640	$102,186
2023 (n=28)	$211,236	$159,849
2024 (n=26)	$187,391	$160,720

n: number

Figure 3.6. Median List and Net Price for Oncology Drugs

Oncology Drugs		
Year	**List Price**	**Net Price**
2022 (n=13)	$357,623	$316,515
2023 (n=15)	$346,980	$302,381
2024 (n=16)	$442,645	$403,823

n: number

Figure 3.7. Median List and Net Price for Endocrine/Metabolic Drugs

Endocrine/Metabolic Drugs		
Year	**List Price**	**Net Price**
2022 (n=6)	$419,876	$391,835
2023 (n=8)	$476,867	$439,664
2024 (n=6)	$781,220	$742,159

n: number

Health System Impact Analysis

This analysis focused on drugs approved between 2022 and 2024 that were previously reviewed by ICER. Of the 25 ICER-reviewed drugs, Paxlovid was excluded from the scope (see above). We also excluded Amvuttra (Vutrisiran) from this analysis because the indication for the ICER review and the first FDA approval were different.

For the remaining 23 ICER-reviewed drugs, we compared their net prices with ICER's HBPB (See Table 3.4). ICER's HBPB suggests a price range, net of any discounts and rebates, that aligns fairly with the overall health benefits the treatment provides for patients over their lifetime, based on the data available at the time of drug approval. Prices at or below these thresholds help ensure that the health benefits gained by patients using new treatments are not outweighed by health losses due to long-term cost pressures that lead individuals to delay care, abandon care, or lose health insurance. For drugs with net prices above ICER's HBPB, we estimated the excess drug spending in the first year post-approval attributable to pricing above the HBPB. We then contextualized the consequences of this first-year excess drug spending by estimating the health benefits foregone due to above-benchmark pricing (i.e., health opportunity costs). We estimated these health opportunity costs through three approaches: (1) equal value life years (evLYs) lost, (2) health insurance coverage loss and associated mortality, and (3) additional number of people that could gain access to high-value drugs if excess spending were redirected.

Table 3.4. ICER-Reviewed Drugs (N=23)

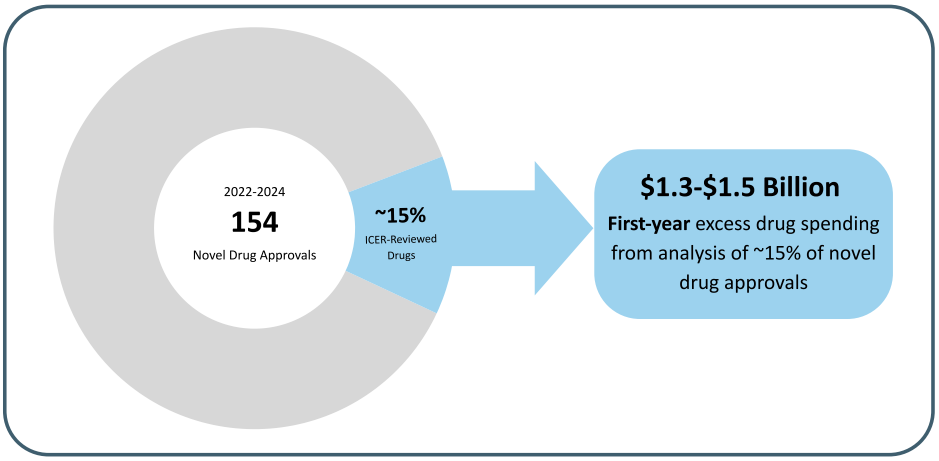
Drug Name	Condition*	Annual List Price	Annual Net Price (Best Estimate)	ICER HBPB‡	Net Price within ICER HBPB	Discount Needed to meet HBPB§
Approved in 2022						
Cibinqo	Atopic Dermatitis	$59,787	$33,720	$30,600 ~ $41,800	Yes	--
Carvykti†	Multiple Myeloma	$465,000	$465,000	$230,000 ~ $312,000	No	32.9% ~ 50.5%
Camzyos	Hypertrophic Cardiomyopathy	$89,500	$64,127	$12,000 ~ $15,000	No	76.6% ~ 81.3%
Mounjaro	Type 2 Diabetes	$12,666	$4,582	$5,500 ~ $5,700	Yes	--
Zynteglo†	Beta Thalassemia	$2,800,000	$2,138,693	$2,120,000 ~ $2,770,000	Yes	--
Relyvrio#	Amyotrophic Lateral Sclerosis	$163,002	$130,401	$9,100 ~ $30,700	No	76.5% ~ 93%
Hemgenix†	Hemophilia A and B	$3,500,000	$2,646,633	$2,930,000 ~ $2,960,000	Yes	--
Adstiladrin†	Bladder Cancer	$240,000	$239,643	$158,600 ~ $262,000	Yes	--
Briumvi	Relapsing Forms of Multiple Sclerosis	$98,333	$66,445	$16,500 ~ $34,900	No	47.5% ~ 75.2%
Approved in 2023						
Leqembi	Early Alzheimer's Disease	$26,500	$26,491	$8,900 ~ $21,500	No	18.8% ~ 66.4%
Veozah	Vasomotor Symptoms from Menopause	$6,692	$3,998	$2,000 ~ $2,600	No	35% ~ 50%
Roctavian†	Hemophilia A & B	$2,446,875	$2,446,875	$1,960,000 ~ $1,960,000	No	19.9% ~ 19.9%
Fabhalta	Paroxysmal Nocturnal Hemoglobinuria	$550,000	$462,000	$178,000 ~ $180,000	No	61% ~ 61.5%
Casgevy†	Sickle Cell Disease	$2,200,000	$2,200,000	$1,350,000 ~ $2,050,000	No	6.8% ~ 38.6%
Lyfgenia†	Sickle Cell Disease	$3,100,000	$3,100,000	$1,350,000 ~ $2,050,000	No	33.9% ~ 56.5%
Approved in 2024						
Lenmeldy†	Metachromatic Leukodystrophy	$4,250,000	$4,250,000	$2,294,000 ~ $3,940,000	No	7.3% ~ 46%
Attruby	Transthyretin Amyloid Cardiomyopathy	$244,539	$183,404	$13,600 ~ $39,000	No	78.7% ~ 92.6%

Drug Name	Condition*	Annual List Price	Annual Net Price (Best Estimate)	ICER HBPB‡	Net Price within ICER HBPB	Discount Needed to meet HBPB§
Rezdiffra	Metabolic Dysfunction-Associated Steatohepatitis (Non-Alcoholic Steatohepatitis)	$48,058	$43,373	$39,600 ~ $50,100	Yes	--
Winrevair	Pulmonary Arterial Hypertension	$245,140	$196,112	$17,900 ~ $35,400	No	81.9% ~ 90.9%
Voydeya	Paroxysmal Nocturnal Hemoglobinuria	$50,261	$38,390	$12,300 ~ $13,100	No	65.9% ~ 68%
Ohtuvayre	Chronic Obstructive Pulmonary Disease	$35,400	$35,400	$7,500 ~ $12,700	No	64.1% ~ 78.8%
Rytelo	Anemia in Myelodysplastic Syndrome	$354,781	$352,115	$94,800 ~ $113,000	No	67.9% ~ 73.1%
Cobenfy	Schizophrenia	$22,508	$18,007	$16,000 ~ $20,000	Yes	--

HBPB: Health Benefit Price Benchmark, ICER: Institute for Clinical and Economic Review
*Indication as described in the ICER evidence reports
†One-time administered cell or gene therapies
‡ICER's HBPB is presented as a range
§Discount from net price required to reach ICER HBPB for drugs priced above its upper bound
#We acknowledge that Relyvrio was withdrawn from the market in 2024 based on negative Phase III results. As our focus was on price at launch, we included this drug in our analysis because it was on the market for over a year.

Excess Drug Spending

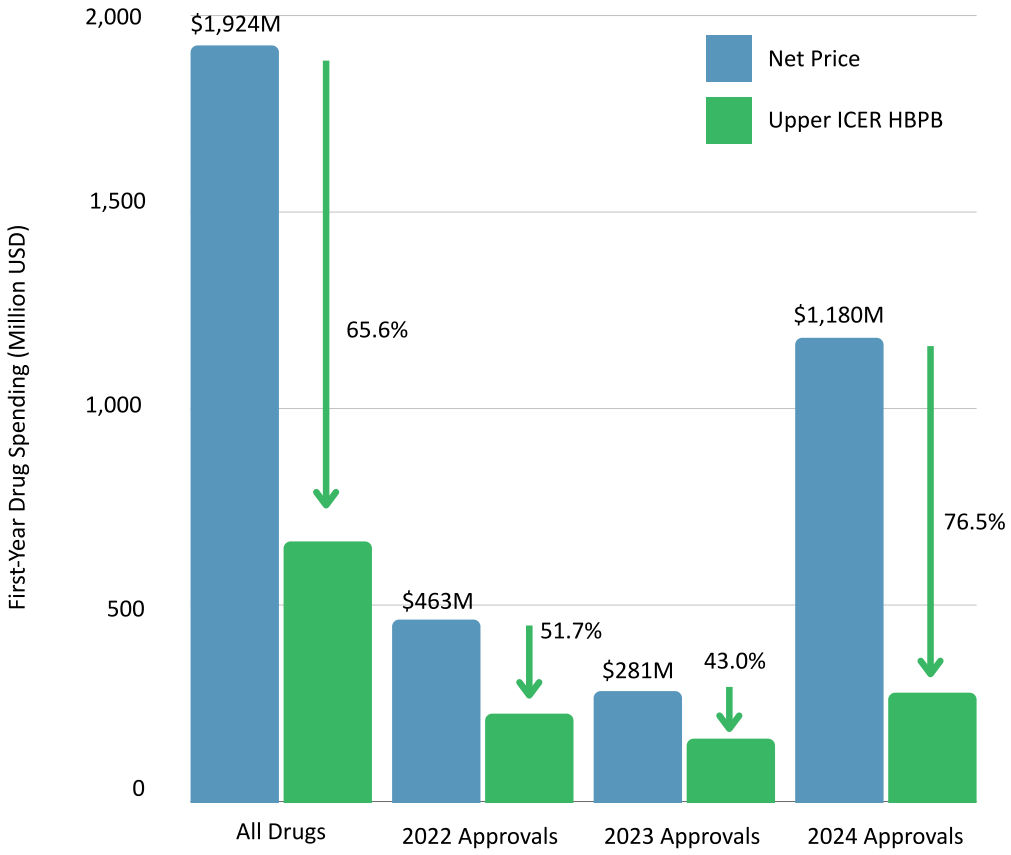
Sixteen of 23 ICER-reviewed drugs in scope (70%) had annual net prices exceeding ICER's upper-bound HBPB. For these 16 drugs, the estimated first-year drug spending was $1.92 billion, while expected spending at ICER's HBPB bounds would have been $431–661 million. This yielded first-year excess drug spending of $1.26–$1.49 billion due to pricing above ICER's HBPB, representing 66%–78% of the estimated first-year spending on these drugs.



When stratified by approval year (see Figure 3.8 and Table 3.5 below), drugs approved in 2024 had the highest first-year excess spending of $903–$1,020 million, representing 68%–71% of total excess drug spending across all 16 drugs. Winrevair (sotatercept-csrk), a drug for pulmonary arterial hypertension, accounted for 41%–44% ($555–615 million) of total excess spending across all 16 drugs. The share of total excess spending for the other drugs ranged from 0.1% to 9.8%.

We found that the five gene and cell therapies (Carvykti [ciltacabtagene autoleucel], Roctavian[valoctocogene roxaparvovec-rvox], Casgevy [exagamglogene autotemcel], Lyfgenia [lovotibeglogene autotemcel], and Lenmeldy [atidarsagene autotemcel]) accounted for a relatively small share of total excess spending compared to non-gene and cell therapies (see Table 3.5). Each gene and cell therapy represented less than 1% of total excess spending except for one (Carvykti), and their combined excess spending was only 5%–8% ($75–120 million) of the total. The relatively low share of total excess spending likely reflects either smaller deviations from ICER HBPB, less utilization volume, or a combination of both, compared to non-gene and cell therapies.

Figure 3.8. Relative Reduction in First-Year Drug Spending with ICER HBPB vs. Net Price



HBPB: health benefit price benchmark, M: million, USD: United States Dollars

Table 3.5. Excess First-Year Drug Spending

Drug	Estimated Drug Spending with the Actual Net Price*	Expected Drug Spending with ICER HBPB†	Excess Drug Spending†	Share of Total Excess Spending‡
All drugs (N=16)	**$1,924M**	**$431M ~ $661M**	**$1,264M ~ $1,493M**	**100%**
Approved in 2022 (N=4)	**$463M**	**$146M ~ $223M**	**$240M ~ $317M**	**19% ~ 21.3%**
Carvykti	$203M	$100M ~ $136M	$67M ~ $103M	5.3% ~ 6.9%
Camzyos	$96M	$18M ~ $22M	$74M ~ $78M	5.2% ~ 5.8%
Relyvrio§	$75M	$5M ~ $18M	$58M ~ $70M	4.6% ~ 4.7%
Briumvi	$89M	$22M ~ $47M	$42M ~ $67M	3.3% ~ 4.5%
Approved in 2023 (N=6)	**$281M**	**$126M ~ $160M**	**$121M ~ $155M**	**9.6% ~ 10.4%**
Leqembi	$27M	$9M ~ $22M	$5M ~ $18M	0.4% ~ 1.2%
Veozah	$85M	$43M ~ $55M	$30M ~ $42M	2.4% ~ 2.8%
Roctavian	$11M	$9M ~ $9M	$2M ~ $2M	0.1% ~ 0.2%
Fabhalta	$129M	$50M ~ $50M	$79M ~ $79M	5.3% ~ 6.2%
Casgevy	$17M	$10M ~ $16M	$1M ~ $7M	0.1% ~ 0.4%
Lyfgenia	$12M	$5M ~ $8M	$4M ~ $7M	0.3% ~ 0.5%
Approved in 2024 (N=6)	**$1,180M**	**$160M ~ $278M**	**$903M ~ $1,020M**	**68.3% ~ 71.4%**
Lenmeldy	$4M	$2M ~ $4M	$0M ~ $2M	0% ~ 0.1%
Attruby	$147M	$11M ~ $31M	$116M ~ $136M	9.1% ~ 9.1%
Winrevair	$677M	$62M ~ $122M	$555M ~ $615M	41.2% ~ 43.9%
Voydeya	$12M	$4M ~ $4M	$8M ~ $8M	0.5% ~ 0.6%
Ohtuvayre	$185M	$39M ~ $66M	$119M ~ $146M	9.4% ~ 9.8%
Rytelo	$155M	$42M ~ $50M	$105M ~ $113M	7.6% ~ 8.3%

HBPB: health benefit price benchmark, ICER: Institute for Clinical and Economic Review, M: million, N: number

*The actual US net sales were used as a proxy for the estimated drug spending with the actual net price, and thus reflects the actual utilization volume during the first year. Data on first-year US net sales were obtained from SSR Health, Biomedtracker, companies' financial reports, or IPD Analytics.

†Presented as a range based on the upper and lower ICER HBPB values.

‡Calculated as excess drug spending for each drug or drug group divided by total excess drug spending ($1,263M ~ $1,492M)

§We acknowledge that Relyvrio was withdrawn from the market in 2024 based on negative Phase III results. As our focus was on price at launch, we included this drug in our analysis because it was on the market for over a year.

Opportunity Costs

1. Lost EvLYs:

To understand the health opportunity cost of excess drug spending, we estimated the number of evLYs that could have been generated by redirecting the excess spending. Assuming that each $100,000 spent is equivalent to an evLY lost, the first-year excess drug spending of $1.26–1.49 billion translates to 12,636–14,931 evLYs lost – health benefits lost because the excess drug spending was not directed to other high-value interventions or services. In other words, overspending on 16 of the 23 drugs reviewed in the first year post-launch resulted in more than 12,000 fewer life years in full health than could have been achieved with optimal spending across the US population. Given that in the US, quality-adjusted life expectancy at birth is approximately 64 years, this foregone health benefit is equivalent to the entire healthy lifespan of approximately 200 people.[24]

Table 3.6. Opportunity Costs: Lost evLYs

Drug	Lost evLYs
All drugs (N=16)	12,636 ~ 14,931
Approved in 2022 (N=4)	2,400 ~ 3,174
Approved in 2023 (N=6)	1,210 ~ 1,553
Approved in 2024 (N=6)	9,026 ~ 10,204

evLY: equal value life year, N: number

2. Health Insurance Coverage Loss and Associated Mortality:

We also estimated how the first-year excess spending could translate into loss of insurance coverage and resulting deaths across the US population. Based on published evidence in the literature,[25] we assumed that the excess drug costs are fully passed on to all US enrollees as premium increases. Accounting for how premium increases affect insurance enrollment, we estimated that 97,395–115,080 individuals are expected to lose coverage due to the one-year overspending on the drugs reviewed by ICER. Based on data linking insurance loss to mortality, the estimated coverage loss would result in 351–415 deaths.

In a scenario analysis, we examined alternative assumptions where only a fraction of the excess costs, such as 25%, 50%, or 75%, are passed on as premium increases. The analysis showed that the number of individuals losing insurance coverage, along with the resulting deaths, was directly proportional to the share of excess drug costs that were passed through as premium increases. For instance, if 50% of the excess drug costs are passed through, both the estimates for individuals losing coverage and the associated deaths are effectively halved.

To account for uncertainty in the data used to estimate opportunity costs, we conducted a probabilistic sensitivity analysis (PSA) with simultaneous variation of all opportunity cost parameters. The varied parameters and their ranges are provided in Appendix A2.2, and the full PSA results are presented in Appendix A2.3.

Table 3.7. Opportunity Costs: Coverage Loss

Drug	Number of People Losing Insurance*	Number of Deaths Due to Insurance Loss*
All Drugs (N=16)	97,395 ~ 115,080	351 ~ 415
Approved in 2022 (N=4)	18,501 ~ 24,462	67 ~ 88
Approved in 2023 (N=6)	9,324 ~ 11,966	34 ~ 43
Approved in 2024 (N=6)	69,570 ~ 78,652	251 ~ 283

N: number

*Estimates assume excess drug costs are fully passed through to all US enrollees as premium increases. If only a fraction of excess costs are passed through, estimates decrease proportionally.

3. Additional Access:

Table 3.8 presents the additional number of individuals who could have gained access to a high-valued drug if all the first-year excess spending ($1.26–$1.49 billion) were redirected to that drug. Of the 23 ICER-reviewed drugs, we identified seven high-value drugs priced within ICER's HBPB (Table 3.4). We found that redirecting excess drug spending from the drugs priced above ICER's HBPBs could have instead provided additional access to high-value drugs, ranging from approximately 477–564 individuals for Hemgenix (etranacogene dezaparvovec-drlb) to 275,779–325,854 for Mounjaro (tirzepatide) in a given year.

Table 3.8. Additional Drug Access from Redirected Excess Drug Spending

Drug Names	Condition	Additional Number of Individuals Gaining Access per Year*
Cibinqo	Atopic Dermatitis	37,474 ~ 44,279
Mounjaro	Type 2 Diabetes	275,779 ~ 325,854
Zynteglo	Beta Thalassemia	591 ~ 698
Hemgenix	Hemophilia A and B	477 ~ 564
Adstiladrin	BCG-Unresponsive, Non-Muscle Invasive Bladder Cancer	5,273 ~ 6,230
Rezdiffra	Metabolic Dysfunction-Associated Steatohepatitis	29,134 ~ 34,424
Cobenfy	Schizophrenia	70,176 ~ 82,918

*Additional number of individuals who could gain access to each drug if the excess drug spending ($1,264M ~ $1,493M) were redirected, in addition to those who already have access. Presented as a range corresponding to the ICER's lower and upper HBPB.

Manufacturer Price Justification

Manufacturers often provide justification for the pricing of their drugs. We requested manufacturer input on their pricing justification for each drug in scope reviewed by ICER.

Of the 16 drugs with net prices above ICER's HBPB, 69% did not submit information on pricing justification (11 drugs). Of those that did provide pricing justification, we categorized the pricing justification into six categories. Table 3.9 presents pricing justification for each drug. (See Appendix A2.5 for examples of the price justification for each category).

Manufacturers mentioned alignment with clinical and economic value for nearly all drugs, although the specifics of how this is achieved are not always clear. Most drugs had more than one category of pricing justification. One manufacturer cited their own internal economic models for justification.[26] We also conducted our own search for pricing justification from online sources. Details of this search and the results can be found in Appendix A2.5.

Eisai's Pricing Justification for Leqembi (lecanemab-irmb)[26]

Eisai's pricing approach for Leqembi included a transparent use of evidence and cost-effectiveness analysis to justify the price, shifting the discussion from what the price justification is to alignment on underlying model assumptions and methods.

Table 3.9. Categories for Pricing Justification Submitted by Manufacturers: Individual Drug Data

Drug	Aligned with Clinical and Economic Value	Novelty	Clinical and Safety Profile	Promote Patient Access	Funding Future Research	Internal Economic Model	Priced in Alignment with Competing Products	No Comment
Veozah	★	★	★					
Winrevair	★	★	★	★	★			
Leqembi	★			★		★		
Lenmeldy	★							
Attruby							★	
Carvykti								⃠
Camzyos								⃠
Relyvrio*								⃠
Briumvi								⃠
Roctavian								⃠
Fabhalta								⃠
Casgevy								⃠
Lyfgenia								⃠
Voydeya								⃠
Ohtuvayre								⃠
Rytelo								⃠

*We acknowledge that Relyvrio was withdrawn from the market in 2024 based on negative Phase III results. As our focus was on price at launch, we included this drug in our analysis because it was on the market for over a year.

3.2. Discussion

The aim of this report is to inform current policy conversations that address launch prices, and highlight opportunities to enhance affordability and access. Recent reports have suggested a flattening or decrease in the net prices of brand-name drugs.[27,28] However, these analyses encompass all drugs, not just newly launched drugs, and do not typically assess prices on a drug-by-drug basis. The lack of data has hampered efforts to understand and address launch prices.

Our evaluation of launch prices of newly approved drugs over the past three years contributes new analyses to this important policy question. This report shows that prescription drug launch prices continue to rise at a rate that exceeds inflation, gross domestic product (GDP) growth, and overall health care cost growth. Specifically, our analysis indicates that the inflation-adjusted annual list launch price of drugs increased by 24% from 2022 to 2024, while the annual net price estimate saw a larger increase of 51% during the same period. To foster innovation, there is generally a willingness to assign higher prices to treatments that provide a substantial benefit over prior options, as this rewards innovation. Therefore, higher launch prices may reflect the introduction of more innovative therapies in recent years. We investigated the relationship between various drug characteristics and launch prices and found that gene and cell therapies, orphan products, first-in-class drugs, and certain therapeutic areas—such as oncology and endocrine/metabolic drugs—were all associated with higher launch prices. However, after adjusting for these characteristics, the inflation-adjusted launch price of drugs still increased by 25% per year for the list price and 33% for the net price, suggesting that the rise cannot be solely attributed to these product factors.

In recent years, several federal policies have been implemented to curb the rising costs of prescription drugs, which are a key driver of health spending. For example, under the Inflation Reduction Act of 2022 (IRA), the Centers for Medicare and Medicaid Services (CMS) has initiated drug price negotiations with the manufacturers of certain high-cost drugs to reduce prices of Medicare's highest-spending medications starting in 2026.[29] Some have suggested that the anticipation of negotiations may lead manufacturers to increase initial launch prices in preparation for future price reductions, particularly for drugs aimed at the Medicare population.[13,14] Given the timeframe of this analysis, we are establishing a baseline to measure future trends in launch price to assess how and if negotiated Medicare prices going into effect in 2026 are impacting launch prices.

Pricing to value, or ensuring that the launch price is tied to how much benefit it provides to patient, is a commonly used approach outside the US, and results in substantially lower prices of some brand-name drugs in countries that negotiate prices based on the value of the drugs.[30,31] Consequently, a proposal to use a most favored nation (MFN) pricing policy, an approach that would allow the US to benefit from the lower prices negotiated in other countries, has been put forward.[32,33] However, this approach presents many operational challenges, particularly its reliance on foreign benchmarks that may not reflect the financial considerations and societal values of the

US population. ICER's approach to estimating the value of a drug is grounded in those considerations. ICER's HBPB, which is the price range, net of any discounts and rebates, that aligns fairly with the overall health benefits the treatment provides based on the data available at the time of drug approval, is scaled to reflect spending that does not cause harmful effects on patients through higher health insurance premiums.

Our analysis found that approximately 70% of the 23 drugs reviewed by ICER that were approved between 2022 and 2024 (~15% of the 154 novel approvals during that timeframe) had net prices above ICER's HBPB. Aligning prices of these therapies with ICER's HBPB could have saved the US health care system approximately $1.3 to $1.5 billion in the first year post-approval alone – savings that could have been redirected to higher-value drugs and services. For example, our analysis showed that overspending on therapies in their first year post-launch resulted in more than 12,000 fewer life years in full health than could have been achieved with optimal spending across the US population, and redirecting this excess spending could have prevented over 97,000 individuals from losing health insurance due to premium increases. We also showed that the one-year overspending from these drugs directed to other high-value drugs could provide many individuals with currently uncovered prescriptions access to high-value therapies such as Mounjaro (tirzepatide), Zynteglo (betibeglogene autotemcel), or Cobenfy (xanomeline and trospium chloride). Since launch prices typically remain above value-based benchmarks until generics or biosimilars enter the market, this excess spending is likely to accumulate over a decade for each drug, and our first-year estimates represent only a fraction of the total lifecycle excess spending. While factors beyond cost-effectiveness influence drug pricing, our findings highlight the need for greater attention to pricing drugs to value and to the allocation of limited health care resources.

There are important limitations to consider in our analysis. One area of uncertainty is the net price estimates we used. While we relied on the best available sources for net price estimates at launch, actual net pricing data are not transparent, and our estimates may therefore differ from the true net price. To mitigate these uncertainties, we allowed drug manufacturers whose drugs were included in the excess drug spending analysis to correct net price estimates and provide other key data points. Only two manufacturers provided us with their net price (Ferring Pharmaceuticals for their drug Adstiladrin [nadofaragene firadenovec-vncg] and CSL Behring for their drug Hemgenix [etranacogene dezaparvovec-drlb]). It is also important to consider the estimated excess drug spending within the context of the specified time frame—the first year following FDA approval. We took this approach because net prices can change over time, and analyzing a longer time period may lead to different results. Additionally, ICER's HBPB is based on the available evidence on the drug at the point of drug launch. New data may become available post-launch, potentially changing the price benchmark. We also note that our analysis of excess drug spending for recently approved drugs (those launched in mid to late 2024) may be more uncertain than the others, as first-year net sales data are not yet fully available for these recently approved drugs. In the absence of full-year net sales data, first-year net sales were estimated by applying a multiplier to the available partial

sales data, assuming net sales were constant throughout the year. This is likely to be a conservative assumption as it does not account for an increased uptake. Also, the estimated evLYs lost due to excess drug spending is largely dependent on the assumed health opportunity costs in the US. While our assumption ($100,000 per evLY) aligns with the cost-effectiveness thresholds commonly used in the US and with a simulation study estimating health opportunity costs in the US population, no formally established or standardized health opportunity cost exists in the US.[25] Finally, the impact of excess drug spending on coverage loss and associated mortality depends on several uncertain parameters, such as the elasticity of insurance loss with respect to premium changes and the number needed to lose insurance to cause one death. We conducted a probabilistic sensitivity analysis, which is presented in Appendix A2.2, to address parameter uncertainty.

These analyses, even with its important limitations, contributes to the critical decisions facing the US health care system. Importantly, more transparency on net prices and more communication from drug makers on how prices are chosen (see call-out box about Eisai and Leqembi) will help policymakers address affordability with evidence. Health insurance premiums are rising at unsustainable rates, and significant increases in the number of uninsured are expected. By calculating the net price trends of pharmaceutical products, we aim to help identify opportunities for creating more affordable health insurance access for patients.

4. Patient Access: 2024 Launches

We aimed to evaluate key barriers to access of newly approved drugs in 2024, including coverage restrictions as described in coverage policies, prior authorization burdens, and patient cost-sharing, based on real-world patient access for 54 novel drugs approved in 2024 and one additional drug of interest (Zepbound for obesity). However, due to lack of data availability, we were unable to assess whether coverage restrictions were consistent with ICER's Cornerstones of "Fair" Drug Coverage criteria, and therefore, we only report on coverage policy availability. Furthermore, our data are from commercial payers only, and thus our report reflects that segment of the payer landscape. Finally, our data sources did not have all drugs in scope; therefore, we were unable to evaluate all drugs in scope. Table 4.1 provides information on the number of drugs we evaluated in each section. We also conducted facilitated group discussions with patient advocacy groups to discuss access challenges, in partnership with the National Health Council, and summarized the results in Subsection 4.4.

Table 4.1. Data Availability for Access Databases

Subsections	Number of Drugs Evaluated
Initial Coverage (Subsection 4.1.)	24 + 1 (Zepbound – see Section 5)
Prior Authorization Burdens (Subsection 4.2.)	17 + 1 (Zepbound – see Section 5)
Patient Cost Sharing (Subsection 4.3.)	17 + 1 (Zepbound – see Section 5)
Patient Voices on Access Challenges (Subsection 4.4.)	8 drugs previously reviewed by ICER

4.1. Initial Coverage Policy Availability

To evaluate insurance coverage for newly approved drugs in 2024, we obtained data from the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) database, which includes information on specialty drug coverage decisions issued by up to 18 large US commercial health plans. Data reported from the SPEC database have two data cutoff dates: December 2024 and April 2025.

At the time of the data cutoff date in December 2024, 18 of the 56 drugs approved in 2024 had policies available for analysis in the SPEC database; by April 2025, 24 of the 56 drugs had available policies. The absence of a policy does not necessarily imply payer non-coverage.

Table 4.2 summarizes coverage policy availability for all drugs found in the database at both cutoff points. As of December 2024, coverage policy availability ranged from 0% to 89% for the drugs in scope. Drugs approved in the first half of 2024 were more likely to have policies available. In contrast, for the majority of drugs approved in the second half of 2024, coverage policies were not found for about half of the payers. No coverage policies were found at all for six drugs, all of which

were approved in the fourth quarter of 2024. By April 2025, policy availability had increased, ranging from 28% to 89% for the drugs in scope.

For many drugs, there appeared to be at least a six-month delay in posting coverage policies, even for first-in-class drugs where no other treatments were previously available. For example, Miplyffa (arimoclomol), the first treatment approved for those with Niemann-Pick disease, type C, was approved in September 2024. Although only 11% of available policies explicitly covered the drug by December 2024, policy coverage rose to 50% by April 2025. There were six drugs where less than 50% of payers had policies available by April 2025, of which five were approved in the second half of 2024. The sixth drug (Ohtuvayre [ensifentrine]), however, was approved by the FDA in June 2024 for adults with chronic obstructive pulmonary disease (COPD). Only 17% of available policies explicitly covered Ohtuvayre by December 2024; that number increased slightly to 28% by April 2025.

Of the 24 drugs in scope, only two had available coverage policies explicitly indicating non-coverage as of April 2025: 1) Kisunla (donanemab-azbt], approved by the FDA for Alzheimer's disease in July 2024, was explicitly not covered by 33% of policies, and 2) Alyftrek (vanzacaftor/tezacaftor/deutivacaftor) approved by the FDA for cystic fibrosis in December 2024, was explicitly not covered by 6% of payers.

On the other hand, the therapies with the highest rate of available coverage policies, indicating coverage, relative to the other drugs, are gene or cell therapies. For example, although both Lenmeldy (atidarsagene autotemcel) for metachromatic leukodystrophy and Beqvez (fidanacogene elaparvovec-dzkt) for Hemophilia B are gene therapies with high prices, over 80% of payer policies indicated coverage of these therapies. For Amtagvi (lifileucel), a cell therapy for the treatment of melanoma, almost 90% of plans had coverage of the drug. It is important to note that all three therapies were approved in the first quarter of 2024.

Table 4.2. Coverage Policies Availability in the SPEC Database

Drug	Condition	Approval Date	Data Cutoff: December 2024			Data Cutoff: April 2025		
			Coverage Policies†			Coverage Policies†		
			Policy Not Found‡ (n=18)	Drug Not Covered (n=18)	Drug Covered (n=18)	Policy Not Found‡ (n=24)	Drug Not Covered (n=24)	Drug Covered (n=24)
Amtagvi	Melanoma	2/16/2024	11%	-	89%	11%	-	89%
Rezdiffra	MASH	3/14/2024	28%	-	72%	28%	-	72%
Lenmeldy	MLD	3/18/2024	11%	-	89%	6%	-	94%
Duvyzat	DMD	3/21/2024	44%	-	56%	39%	-	61%
Winrevair	PAH	3/26/2024	28%	-	72%	22%	-	78%
Voydeya	PNH	3/29/2024	50%	-	50%	39%	-	61%
Beqvez	Hemophilia B	4/25/2024	17%	-	83%	17%	-	83%
Xolremdi	WHIM syndrome	4/26/2024	44%	-	56%	28%	-	72%
Rytelo	Myelodysplastic syndromes	6/6/2024	44%	-	56%	44%	-	56%
Iqirvo	Primary biliary cholangitis	6/10/2024	67%	-	33%	50%	-	50%
Piasky	PNH	6/20/2024	39%	-	61%	28%	-	72%
Ohtuvayre	COPD	6/26/2024	83%	-	17%	72%	-	28%
Kisunla	Alzheimer's disease	7/2/2024	39%	28%	33%	28%	33%	39%
Tecelra	Synovial sarcoma	8/1/2024	44%	-	56%	39%	-	61%
Livdelzi	Primary biliary cholangitis	8/14/2024	67%	-	33%	39%	-	61%
Ebglyss	Atopic dermatitis	9/13/2024	100%	-	-	67%	-	33%
Miplyffa	Niemann-Pick disease type C	9/20/2024	89%	-	11%	50%	-	50%
Vyloy	Stomach cancer	10/18/2024	100%	-	-	44%	-	56%
Aucatzyl	Leukemia	11/8/2024	94%	-	6%	39%	-	61%
Kebilidi	AADC deficiency	11/13/2024	94%	-	6%	50%	6%	44%
Revuforj	Leukemia	11/15/2024	100%	-	-	50%	-	50%
Tryngolza	FCS	12/19/2024	100%	-	-	61%	-	39%
Alhemo	Hemophilia A/B	12/20/2024	100%	-	-	50%	-	50%
Alyftrek	Cystic fibrosis	12/20/2024	100%	-	-	67%	-	33%

#: number, AADC: Aromatic L-amino acid decarboxylase, COPD: Chronic obstructive pulmonary disease, DMD: Duchenne muscular dystrophy, FCS: familial chylomicronemia syndrome, MASH: Metabolic dysfunction-associated steatohepatitis, MLD: Metachromatic leukodystrophy, PAH: Pulmonary arterial hypertension, PNH: Paroxysmal nocturnal hemoglobinuria, WHIM: warts, hypogammaglobulinemia, infections and myelokathexis

*Price was rounded to the nearest dollar amount

†Percentages are based on the 18 potential policies for each drug

‡Payer did not have a publicly available coverage policy

4.2. Prior Authorization Burden

We obtained first quarter 2025 data from IQVIA's Longitudinal Access and Adjudication Data (LAAD), which contains prior authorization analytics and patient cost-sharing information for pharmacy claims made to commercial insurances and out-of-pocket costs for cash-paid prescriptions. First quarter 2025 data were available for 28 of the 54 drugs in scope for the 2024 approval year. We excluded 11 of 28 drugs that had less than 100 total commercial written prescriptions for the quarter to ensure numeric trends were not caused by randomness, leaving 17 drugs in the analysis.

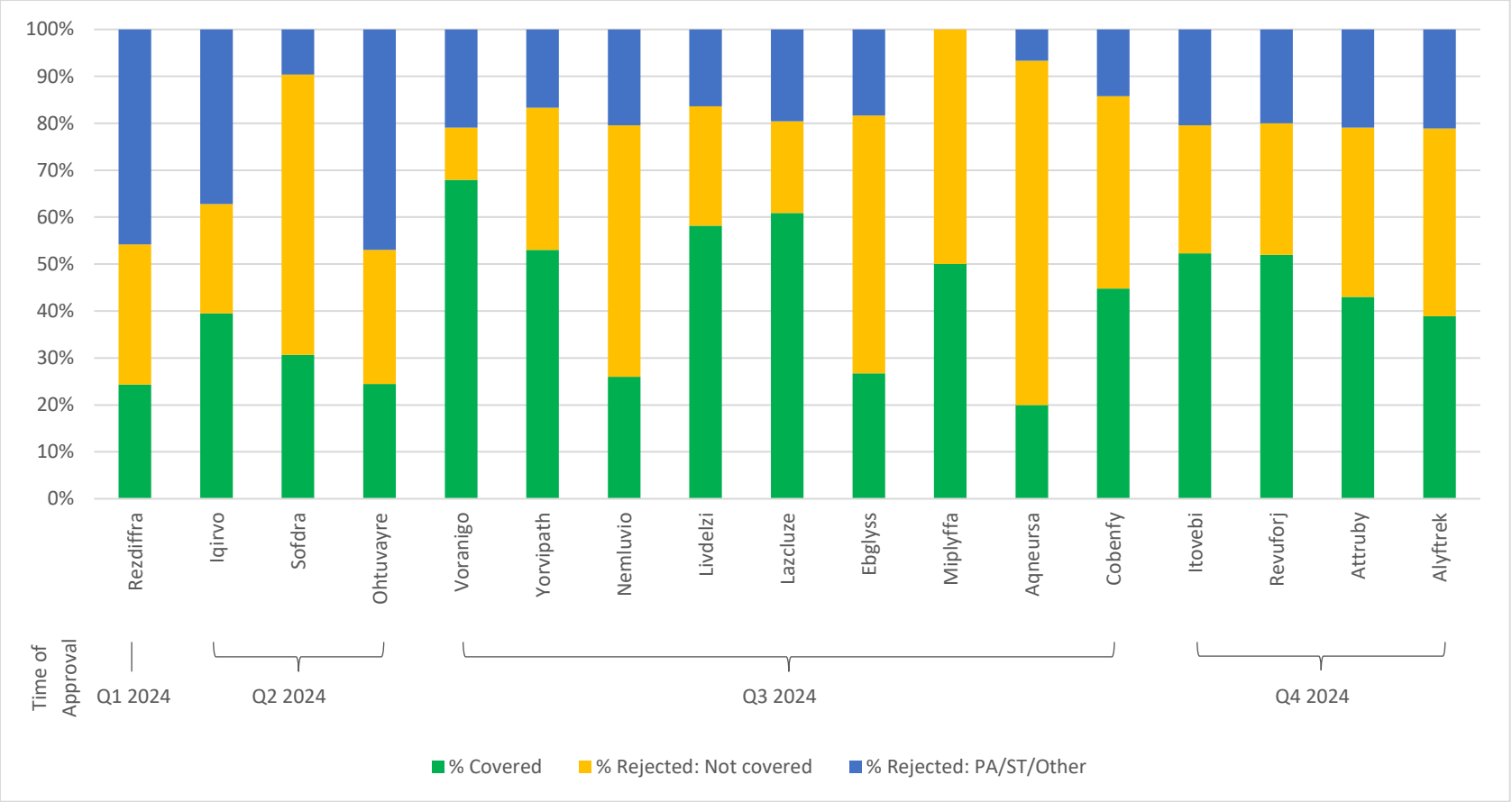
Table 4.3 shows summary metrics for the number of commercial prescriptions written and dispensed for the drugs in scope that were captured in the LAAD database, while Figure 4.1 illustrates the percentage of new-to-brand prescriptions that were covered, not covered, or rejected for all attempts at coverage. New-to-brand claims represent a patient's first prescription of a drug. That prescription can be filled or rejected. Rejection can happen for various reasons, including non-coverage, need for prior authorization, step therapy, or administrative errors. Prescriptions that are rejected may be re-tried multiple times before final adjudication. Finally, prescriptions may be abandoned – i.e., the prescription was filled by the pharmacy, but the patient did not pick it up due to cost or other reasons.

Table 4.3. Prior Authorization Analytics for Commercial Pharmacy Claims in the IQVIA Database (Q1 2025 Data)

Drug Name	Condition	Approval Date	Total Commercial Written Prescriptions	Total Commercial New-to-Brand Dispensed
Rezdiffra	MASH	3/14/2024	6,679	1,158
Iqirvo	Primary biliary cholangitis	6/10/2024	730	164
Sofdra	Primary axillary hyperhidrosis	6/18/2024	1,682	676
Ohtuvayre	COPD	6/26/2024	715	124
Voranigo	Brain cancer	8/6/2024	1,285	189
Yorvipath	Hypoparathyroidism	8/9/2024	152	37
Nemluvio	Prurigo nodularis	8/12/2024	4,588	1,173
Livdelzi	Primary biliary cholangitis	8/14/2024	1,617	327
Lazcluze	Non-small cell lung cancer	8/19/2024	212	36
Ebglyss	Atopic dermatitis	9/13/2024	3,093	706
Miplyffa	Niemann-Pick disease type C	9/20/2024	102	5
Aqneursa	Niemann-Pick disease type C	9/24/2024	207	18
Cobenfy	Schizophrenia	9/26/2024	2,526	694
Itovebi	Breast cancer	10/10/2024	212	57
Revuforj	Leukemia	11/15/2024	120	26
Attruby	ATTR-CM	11/22/2024	218	57
Alyftrek	Cystic fibrosis	12/20/2024	486	211

ATTR-CM: transthyretin amyloid cardiomyopathy, COPD: chronic obstructive pulmonary disease, MASH: metabolic dysfunction-associated steatohepatitis

Figure 4.1. Rate of Commercial New to Brand Prescriptions Covered, Not Covered, or Rejected Across All Attempts – Q1 2025



PA: prior authorization, Q: quarter, ST: step therapy

Graph illustrates the proportion of new-to-brand prescriptions for each drugs that were covered, not covered, or rejected by commercial insurers in the first quarter of 2025. In this illustration, covered is defined as the sum of the percentage of prescriptions that were filled + abandoned. The graph also depicts the calendar quarter of the date of FDA approval below the x-axis.

On average, 42% of total dispensed commercial new-to-brand prescriptions were covered, but only 29% were ultimately successfully filled overall in the first quarter of 2025. There was variation in fill rates, from less than 10% for Miplyffa (arimoclomol) for Niemann-Pick disease, Ohtuvayre (ensifentrine) for COPD, and Rezdiffra (resmetirom) for MASH, to more than 40% for Itovebi (inavolisib) and Lazcluze (lazertinib) for cancer, and Livdelzi (seladelpar) for primary biliary cholangitis. Although we are not able to directly correlate coverage policies with the prescription fill data, we did examine if the prescription fill data matched general coverage policy trends for the drugs where we had data on both coverage policies and prescription fill rates (Alyftrek [vanzacaftor/tezacaftor/deutivacaftor], Iqirvo [elafibranor], Miplyffa [arimoclomol capsules], Ohtuvayre [ensifentrine], Rezdiffra [resmetirom]). For three drugs – Alyftrek, Iqirvo, and Ohtuvayre – prescription fill rates appear to be consistent with what would be expected for the coverage landscape. For example, only 28% of plans had coverage policies for Ohtuvayre as of April 2025; only 5% of new-to-brand prescriptions were filled in the first quarter of 2025.

***Patient Voices on Access**: We heard about other factors outside insurance coverage impacting access, including health system complexity, health inequities, drug burden, and cost-related issues.*

On the other hand, although the majority of commercial payers in the SPEC database appeared to cover Rezdiffra for MASH (72% as of April 2025), prescription fill data show that about three-quarters of prescriptions for Rezdiffra in the first quarter of 2025 were rejected and only 6% were ultimately filled. This discrepancy may speak to both restrictions in coverage (e.g., prior authorization, step therapy) and to barriers to access other than insurance coverage, as told to us by a patient advocacy group:

"One of the issues for our patient base is the requirement of a biopsy to receive the medication. There are some providers that require that, and I don't know anybody who really wants to get a liver biopsy, because that's invasive, it hurts, and it's very costly. But that's definitely a barrier."
– Patient Representative

The majority of commercial new-to-brand prescriptions for newly approved drugs in 2024 were rejected (58%). Rejection rates were greater than 50% regardless of whether the drug was first-in-class, considered an orphan drug, or deemed cost-effective by ICER (Table 4.4). Non-coverage of the drug was the most common reason for rejection, perhaps reflecting that the establishment of coverage policies often significantly lags approval dates. When comparing fill rates by therapeutic area, we found that non-oncology drugs were more likely to be rejected (63% vs. 42%). Oncology treatment tends to be more standardized than other fields, and guidelines also tend to be updated more frequently than other fields, perhaps allowing for better alignment of initial coverage policies with clinical practice. However, non-oncology drugs were a heterogeneous group, and thus, the reasons for rejection may be much more variable. On average, first-in-class drugs were rejected at a

higher rate than those that were not (63% vs. 54%). In contrast, we saw a greater percentage of rejections for non-orphan drugs versus orphan drugs (53% vs. 64%). Orphan drugs represent treatments that are intended for rare disease populations with limited options, and the data seem to suggest that payers could be more relaxed with their coverage requirements for these agents.

Finally, we examined data on prescription abandonment rates. Abandoned claims are prescriptions that were filled by the pharmacy but were not picked up by the patient. Overall, 35% of covered commercial new-to-brand prescriptions were abandoned, though there was variation in abandonment rates from 11% for Sofdra and Alyftrek to 100% for Miplyffa (Table 4.4), though these rates should be interpreted with caution for drugs with few prescriptions written and/or filled. Nevertheless, abandonment rates may signal barriers other than insurance coverage, such as high out-of-pocket costs, high deductibles that have not yet been met, lack of cost transparency, or delays in prior authorization adjudication, that prevent patients from picking up a prescription.

Table 4.4. Proportion of Covered New-to-Brand Prescriptions Written in Q1 2025 Abandoned at the Pharmacy

Drug Name	Total Commercial New-to-Brand Dispensed	Covered Overall	Proportion of Covered that were Abandoned
Rezdiffra	1,158	25%	77%
Iqirvo	164	40%	15%
Sofdra	676	30%	11%
Ohtuvayre	124	25%	82%
Voranigo	189	68%	43%
Yorvipath	37	53%	31%
Nemluvio	1,173	26%	12%
Livdelzi	327	59%	13%
Lazcluze	36	61%	25%
Ebglyss	706	27%	29%
Miplyffa	5	50%	100%
Aqneursa	18	20%	33%
Cobenfy	694	44%	19%
Itovebi	57	52%	22%
Revuforj	26	52%	31%
Attruby	57	43%	38%
Alyftrek	211	39%	11%

Table 4.5. Impact of Key Drug Attributes on Detailed Prior Authorization Analytics for Commercial Pharmacy Claims in the IQVIA Database (Q1 2025 Data)

	Covered			Rejected		
	Filled	Abandoned	Overall	Not Covered	PA/ST/Other	Overall
Therapeutic Area						
Oncology (n=4)	40%	18%	58%	21%	20%	42%
Non-oncology (n=13)	24%	13%	37%	44%	19%	63%
First-in-Class						
Yes (n=7)	21%	16%	37%	43%	21%	63%
No (n=10)	32%	13%	46%	33%	21%	54%
Orphan						
Yes (n=9)	30%	17%	47%	35%	18%	53%
No (n=8)	25%	11%	36%	39%	24%	64%
Priced to Value*						
Yes (n=2)	21%	14%	35%	35%	30%	65%
No (n=2)	16%	18%	34%	32%	34%	66%

n: number, PA: prior authorization, ST: step therapy

*As determined by a drug's net price in relation to the ICER Health Benefit Price Benchmark

4.3. Patient Cost Sharing

> ***Patient Voice on Access:*** *There are programs out there from the manufacturer that can have as little as $0 copay. The problem is, Medicare doesn't qualify for that...An injection can be like $10,000 and they're looking at about 20% of that...it's very difficult for people who are living on a fixed income trying to afford that.*
>
> *-Patient representative, speaking about patient out-of-pocket costs*

An important component of this report was to gain insight into the out-of-pocket costs individuals face. Table 4.6 on the next page shows the monthly patient cost-share for the drugs with data available in IQVIA and paid for by a commercial payer. Cost-sharing metrics are organized into buckets of dollar amounts, from $0 to more than $1,500 per prescription, and reflect the final cost a patient paid for the prescription at the pharmacy. 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. Cost-sharing for prescriptions shipped directly from the manufacturer as part of patient assistance programs is also not included in the out-of-pocket cost data.

Overall, there was a high percentage of $0 cost for many drugs. There are likely multiple reasons for this. Many new drugs have manufacturer assistance programs (e.g., “copay cards”) which allow patients to obtain their first few prescriptions at little to no cost to them, though we are not able to ascertain what proportion of prescriptions used a copay card. For expensive specialty drugs or for drugs treating high-cost conditions, a $0 cost may reflect the fact that patients with high deductible plans met the deductible or out-of-pocket maximum for the plan year.

Other cost-share buckets are presumed to be reflective of a drug’s copay or coinsurance, which are dependent on several factors, including a drug’s tier placement on the formulary, rebate pricing, or copay coupons. For the majority of drugs for which patients incurred a cost of more than $0, the costs were $250 or less. A few drugs had notable proportions (>20%) of prescriptions costing over $250, including Attruby (acoramidis) for ATTR-CM, Ebglyss (lebrikizumab) for atopic dermatitis, and Ohtuvayre (ensifentrine) for COPD. Both Attruby and Ebglyss are most likely covered as specialty drugs, and in many plans, specialty drugs are subject to coinsurance. For example, Attruby has a monthly list price of $20,378 (Table 4.6), and so at 20% coinsurance, each monthly prescription would cost in excess of $4,000.

In addition to commercially-insured claims, data on prescriptions paid for in cash were available. Most drugs did not have a significant number of cash pay prescriptions. For those prescriptions where patients paid cash, the amount paid roughly correlates with the monthly list price (See Appendix).

Table 4.6. Patient Out-of-Pocket Costs Per Prescription for Commercial Pharmacy Claims in the IQVIA Database (Q1 2025 Data)

Drug Name	Indication	Patient OOP Cost (Copay/Coinsurance)*				
		$0	$1-$50	$51-$250	$250-$1500	$1500+
Rezdiffra	MASH	10%	67%	13%	5%	6%
Iqirvo	Primary biliary cholangitis	77%	15%	5%	1%	2%
Sofdra	Primary axillary hyperhidrosis	73%	3%	14%	10%	-
Ohtuvayre	COPD	30%	24%	19%	19%	9%
Voranigo	Brain cancer	42%	44%	9%	2%	3%
Yorvipath	Hypoparathyroidism	20%	64%	12%	1%	3%
Nemluvio	Prurigo nodularis	75%	18%	3%	1%	4%
Livdelzi	Primary biliary cholangitis	65%	10%	16%	3%	5%
Lazcluze	Non-small cell lung cancer	72%	10%	12%	1%	4%
Ebglyss	Atopic dermatitis	15%	48%	7%	7%	23%
Miplyffa	Niemann-Pick disease type C	41%	11%	30%	9%	9%
Aqneursa	Niemann-Pick disease type C	91%	5%	3%	1%	1%
Cobenfy	Schizophrenia	57%	11%	24%	5%	3%
Itovebi	Breast cancer	79%	4%	13%	1%	3%
Revuforj	Leukemia	72%	7%	19%	1%	-
Attruby	ATTR-CM	31%	7%	40%	4%	18%

ATTR-CM: transthyretin cardiac amyloidosis, COPD: chronic obstructive pulmonary disease, MASH: metabolic dysfunction-associated steatohepatitis, OOP: out of pocket

*Percentages are based on total commercial written prescriptions. Dollar amount ranges are based on final payment at the pharmacy, and could include discounts such as copay cards, but excludes other discounts (e.g., manufacturer provided debit card, GoodRx, etc.). Out-of-pocket costs are per prescription.

4.4. Patient Experience

In partnership with the National Health Council, ICER conducted facilitated group discussions with nine total patient representatives to better understand the barriers and facilitators of patient access to drugs that were reviewed by ICER and approved in 2024. Table 4.7 below lists the eight drugs, the disease areas, and patient communities represented in these group discussions. Two group discussions were held. One group discussion focused on drugs for more common diseases, and the other focused on drugs for rare diseases. Additional details about the facilitated group discussion methods are provided in Appendix B4.

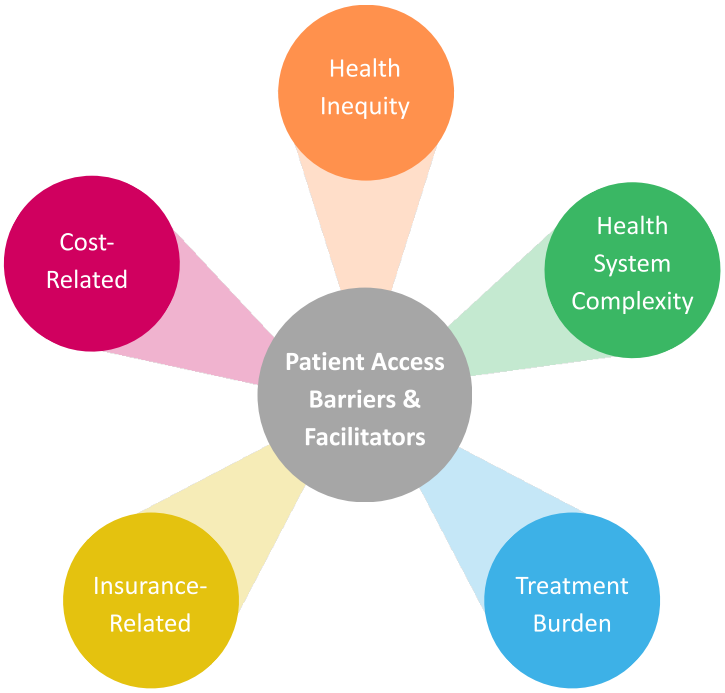
Table 4.7. Eight ICER-Reviewed Drugs that were Approved in 2024

Drug Brand	Approval Date	Disease Area
Rezdiffra	3/14/2024	Metabolic Dysfunction-Associated Steatohepatitis (MASH)
Lenmeldy	3/18/2024	Metachromatic leukodystrophy (MLD)
Winrevair	3/26/2024	Pulmonary arterial hypertension (PAH)
Voydeya	3/29/2024	Paroxysmal nocturnal hemoglobinuria (PNH)
Rytelo	6/6/2024	Anemia in Myelodysplastic Syndrome (MDS)
Ohtuvayre	6/26/2024	Chronic Obstructive Pulmonary Disease (COPD)
Cobenfy	9/26/2024	Schizophrenia
Attruby	11/22/2024	Cardiomyopathy of transthyretin-mediated amyloidosis (ATTR-CM)

Patient Perspectives on Access

Based on the focus group discussions with patient representatives, we categorized the patient access concerns into five key themes: health system complexity, health inequities, drug burden, insurance-related issues, and cost-related issues, as shown in Figure 4.2 and described in detail below.

Figure 4.2. Key Access Themes from the Patient Group Discussions



Health System Complexity

Delay in Diagnosis

We heard from representatives from several conditions – both rare and common - that delays in diagnosis were common, often serving as the first barrier to accessing medications. Delays could be due to both patient and health system factors. For example, PNH and MLD are rare diseases with limited experts in the field, and so individuals with those conditions may display symptoms that are not recognized as part of the disease and may need to travel long distances to seek care. This may contribute to delays in diagnosis and, for MLD, may preclude some children from being eligible for gene therapy. At the other end of the age spectrum, we heard that many people with MDS had signs, symptoms, or lab changes for at least a year before a diagnosis was made.

> *"I think delayed diagnosis, lack of knowledge, and getting to a specialist are probably some of the bigger non-cost related barriers…we found that the majority of patients are treated in the community. They [community physicians] don't see [the condition] as often, and so [patients] may not even be given the opportunity for this medication."*
>
> *-Patient representative, speaking about delays in diagnosis and limited provider knowledge*

Lack of Specialists & Limited Provider Knowledge

For conditions where specialist care is necessary for appropriate diagnosis and/or treatment, a lack of specialists, particularly in rural areas, means the added burdens of time, money, and resources to obtain care. Some of these challenges have been improved through telehealth, but not all. Lack of provider awareness of both disease and treatment options was cited as a barrier to timely treatment, particularly for the COPD and MDS communities. A representative from the COPD community mentioned that access to medications could be improved by increasing awareness of COPD to prescribers and pharmacists alike.

Health Inequities

Social Determinants of Health

Social determinants of health, or non-medical factors that may affect health, play an important role in patients being able to access appropriate care. For example, we heard that people living with schizophrenia may experience unemployment and homelessness, impacting their ability to access and afford treatment that costs more than a few dollars. We also heard that patients who are Black/African American or Latino are more likely to be diagnosed with schizophrenia and yet experience the most barriers to accessing newly available treatments. Families with children who have MLD may have cultural, religious, or language barriers that lead them to delay or choose not to receive care.

> *"Sometimes people are transient. They don't have a lot of income. So cost is always an issue. Where people live is an issue…A lot of the caregivers…are really really eager to get [the patient] on medication…But [on] these medications, you know, they don't really feel good. And so just the nature of the social and community environment…is in some ways what causes the biggest challenge...."*
>
> *-Patient representative, speaking about social and environmental access barriers*

Stigma

Several patient communities mentioned the impact of stigma as a barrier for patients seeking care. As a liver disease, for example, MASH is mistakenly associated with alcohol use, and this stigma can serve as a barrier to access. COPD is often characterized as a "smoker's disease," leading to self-blame and patients feeling guilty to advocate for treatment. For PNH, the stigma is related more to patients seeking pain management for their symptoms, often presenting at the emergency room, where they may experience being labeled as drug seeking.

> *"One of the men that I met has much more severe abdominal pains than I do...He ends up in the hospital needing real pain medication, and they won't give him the pain medications because he's young and they assume he's on drugs and is just looking for a fix.*
>
> *-Patient representative, speaking about the impact of stigma on access to care*

Treatment Burden

A complex method of administration may impair access to treatment, particularly if the treatment needs to be administered in a health care setting. For example, the newly approved gene therapy for MLD requires a three to four-month process of administration at a center of excellence, often requiring families to relocate for treatment, or for parents to remain separated while they juggle work or other childcare responsibilities. The transportation and relocation costs associated with treatment can be significant barriers to initiating treatment. The new treatment for PAH requires refrigeration for storage, and warming and mixing before injection, but limited training is provided to patients and caregivers. Finally, we heard that tests required to access treatment can increase the burden on the patient, which may limit access. For example, diagnosis with MASH and access to Rezdiffra may require a liver biopsy, which can be both invasive and costly, and thus prevent some patients appropriate for therapy from being treated.

Insurance-Related

Type of Insurance

Patient advocates cited instances where the type of insurance had a dramatic effect on the receipt of care. For example, for people with serious mental illness, private insurance may not cover stays in inpatient facilities, which can cost in the tens of thousands of dollars per month. Thus, only people who can afford the high out-of-pocket treatment costs or who qualify for public insurance are able to access care.

Medicare

We heard about additional complexities for navigating governmental insurance programs, particularly Medicare. For example, for certain drugs (e.g., nebulized drugs for COPD) covered under the medical benefit rather than the pharmacy benefit, costs may be subject to coinsurance rather than a copay. This could mean that the patient is responsible for a much larger share of the drug cost, and drugs obtained through Part B are not subject to the $2,000 out-of-pocket maximum that applies to Part D drugs. Additionally, patients covered by Medicare do not have access to manufacturer assistance such as copay cards, which also increases the financial burden of treatment. This is a particular issue for conditions where the majority of the population is older and may be on fixed incomes (e.g., COPD, MDS, ATTR-CM). For example, a large proportion of MDS patients are on Medicare and thus are responsible for 20% co-insurance on a $10,000 drug that is taken indefinitely.

Step Therapy and Prior Authorization

Prior authorization and step therapy can result in substantial barriers to access to newly available treatments. Patient communities are frustrated with the appearance that the restrictions seem to be driven by high costs when insurance plans do not include a drug in their formulary or exclude coverage entirely. In particular, the ATTR-CM, COPD, PNH, and MDS patient communities struggle to access newly available treatments due to step therapy or prior authorization requirements put in place by their insurance plans. For the COPD community, it has been frustrating to note that even effective therapies that have been on the market for a long time are required to go through utilization reviews.

Cost-Related

Patient Assistance Programs

For many drugs, patient assistance programs sponsored by manufacturers or non-profit entities can assist individuals who cannot afford the drug. However, the process of receiving this financial assistance can be cumbersome and has limitations, such as one-time use. For example, one PNH patient received assistance from a manufacturer for $4,000 to cover the first infusion of their treatment, but was then denied any future assistance after the first use. Other communities described how stressful it is to rely on non-profit organizations that have funding for only a limited number of people or have income thresholds that can present a barrier to access. Finally, the financial relief from patient assistance programs can vary depending on whether copay accumulators are in place through a patient's insurance plan; the use of which can impact a patient's health insurance deductible.

> *"The manufacturer does have a patient assistance program. Generally, it is preferable that we go through non-profits that provide copay assistance before those kick in. It's a very stressful couple of days when the spots do open with these charities. It's really the only way the drug is affordable for most people, you know, even a 20% copay on $20,000 is just not something that anyone's going to be doing on a regular basis."*
>
> *-Patient representative, speaking about patient assistance programs*

Other Cost Concerns

For patients with schizophrenia, COPD, and MLD, many different cost-related concerns were shared regarding access to new medications. In the schizophrenia community, we heard of how significant subpopulations who experience unemployment or housing instability are unable to afford medication that costs more than a few dollars. For example, the gene therapy for MLD costs $4.25 million and is currently the most expensive treatment in the world; the price tag alone can deter families from pursuing treatment. Given that there are no longer any clinical trials in progress, patients no longer have a low-cost option to access this therapy, and consequently, families may feel like treatment is out of reach financially. Even if families have insurance coverage for gene therapy, other costs, such as traveling to another city or state, and all the financial and emotional costs that come from relocating temporarily, can be a barrier to accessing treatment. Finally, copay accumulators put in place by insurance companies can exacerbate financial challenges for patients. These accumulators do not count any contributions from patient assistance programs or co-pay cards toward the annual deductible, holding the patient responsible for the full amount of the deductible and increasing financial stress on patients.

> *"So there's other costs…outside of just the direct cost of the treatment...there's time off of work...you have to go in and see your physician, so there's all of these other costs associated outside of just the treatment in and of itself."*
>
> *-Patient representative, discussing potential financial burdens in addition to treatment costs*

4.5. Discussion

In conducting this research, we experienced challenges related to accessing timely and detailed payer policy coverage and prescription fill data on newly launched drugs, and thus, we emphasize the continued need for transparency and data sharing to improve patient access.

We found that for many drugs in scope, insurance coverage policies are lacking even up to one year after approval. This is consistent with a prior study that found a median time to coverage issuance of 209 days.[34] This length of delay may also reflect the impact of new-to-market blocks, which affected more than half of covered lives in 2024.[35] For example, while CMS mandates review of a newly approved drug within 180 days, commercial payers may have a different timeline, with reviews of certain drug categories only taking place once a year. Regardless of the reason, the lack of timely coverage policies could potentially limit or delay access, as patients would need to engage in lengthy exceptions or prior authorization processes without a policy. Some plans have chosen to

issue initial coverage policies that follow the FDA label quickly after approval to minimize delays in access, and then refining those policies at a later time.[36-38]

Due to data limitations, we were not able to determine if coverage policies were in line with fair access principles as outlined in ICER's Cornerstones of "Fair" Drug Coverage white paper with the data we had available to us.[39] The Fair Access criteria lay out appropriate cost-sharing and utilization management policies, to help payers build coverage policies that appropriately guide clinicians and patients towards the most evidence-based, cost-effective treatment options. This is important to understand, particularly in the context of real-world prescription fill rates. For example, we found that 72% of payers covered Rezdiffra for MASH by April 2025; however, only 6% of new-to-brand prescriptions were filled in the first quarter of 2025. Without an assessment of whether coverage policies meet Fair Access criteria, it is impossible to assess whether the low number of filled prescriptions is appropriate, whether payers are unnecessarily constraining access, or if there are other factors contributing to low fill rates. Furthermore, implementation of utilization management (UM) strategies can be burdensome and costly, with an estimated $93 billion spent annually on implementing and navigating UM programs, and contributing to delays in care and physician burnout.[40] Thus, it is in the best interest of all stakeholders to ensure that coverage policies are adhering to Fair Access principles.

Interestingly, gene and cell therapies had among the least barriers to access coverage-wise, with the majority of payers covering those therapies. This is consistent with a recent study showing that less than 2% of coverage policies denied coverage for the 25 currently approved gene and cell therapies, and almost half the coverage policies were in line with the FDA label without further restrictions imposed.[41] However, for drugs obtained at the pharmacy, we found that having a coverage policy did not always predict access to them. Overall, only 29% of new-to-brand prescriptions were filled, and there were drugs that seemingly had high insurance coverage rates but also had high prescription rejection and abandonment rates. There may be multiple reasons for the low overall rate of filled prescriptions and high rejection rates. For example, higher levels of cost-sharing are correlated with higher rates of medication abandonment.[42] We also heard from patient groups that for some conditions, non-health system barriers (e.g., housing instability and lack of employment for persons living with schizophrenia) could play an important role in prescription abandonment rates. The barriers to filling a prescription may have consequences for clinical outcomes for patients – studies have shown that broader coverage of medications is associated with lower rates of exacerbations in multiple sclerosis, for example.[43] Furthermore, after a prescription is rejected, not all patients receive alternate treatment; one study found that less than one-third of patients fill a prescription for an alternative drug.[44] Thus, many patients are at risk of not getting treatment, particularly in therapeutic areas where there may not be many, or any, therapeutic alternatives (e.g., rare diseases).

Patient out-of-pocket costs varied, likely due to the variation in cost-sharing policies that are set by plan sponsors. It is notable that for the commercially insured population, a large proportion of new-to-brand (i.e., first) prescriptions had $0 out-of-pocket cost. However, this does not necessarily reflect ongoing drug out-of-pocket costs – if the cheaper copay was due to manufacturer offers, those are time-limited and thus overall out-of-pocket costs may still present a barrier to affordability. This is particularly true for drugs subject to coinsurance, which comprise a substantial portion of prescriptions for newly approved drugs. Additionally, for patients with high deductible health plans (estimated to be 27% of covered workers in 2024)[45] out-of-pocket costs for a particular prescription fill will depend on whether they have met their deductible for the year. Finally, for patients with plans that include copay accumulators, manufacturer assistance programs like copay cards may end up impacting out-of-pocket costs as the copay cards do not count towards annual deductibles or out-of-pocket maximums. Though these data give us some insight into cost-sharing as a whole, judgements about affordability are difficult to make given that cost-sharing strategies set by plan sponsors are varied and opaque.

There were several limitations to our analysis. First, neither the SPEC nor the IQVIA LAAD databases contained information for all the drugs approved in 2024. Additionally, it is possible that the 18 commercial payers in the SPEC database provide superior or inferior coverage compared with payers outside of the database. Additionally, since approval dates of drugs vary, we were not able to capture the same time frame for data for each drug (i.e., for drugs approved earlier in the year, there was more data because of a larger time period from which to draw data). We were not able to obtain payer-specific prescription data, and thus, the SPEC and IQVIA LAAD data cannot be directly associated with each other. We also did not have access to data that would allow us to assess whether payer coverage policies are in line with ICER's Cornerstones of "Fair" Drug Coverage criteria. Finally, we did not conduct any statistical analyses, but rather focused on reporting trends in the data.

5. Drug of Special Interest: Zepbound

Given the high demand for GLP-1 treatments, which have dramatically altered the landscape of obesity treatment, as well as concerns about access and affordability, we chose to evaluate patient access to Zepbound for the treatment of obesity as an additional drug of interest for this report using the same data sources described in Section 4. The data do not include information on access to Mounjaro, which is indicated for the treatment of Type 2 diabetes, though we acknowledge that Mounjaro may be prescribed off-label for the treatment of obesity. The choice of Zepbound for this analysis, and not another GLP-1 used for obesity, should not be interpreted as an endorsement of one GLP-1 over another. Instead, we chose Zepbound as it is the most recently-launched GLP-1 for obesity, and the aim of this report is to look at drugs recently launched. Given that both Zepbound and Wegovy are cost-effective for the treatment of obesity, these results are likely representative of both drugs.

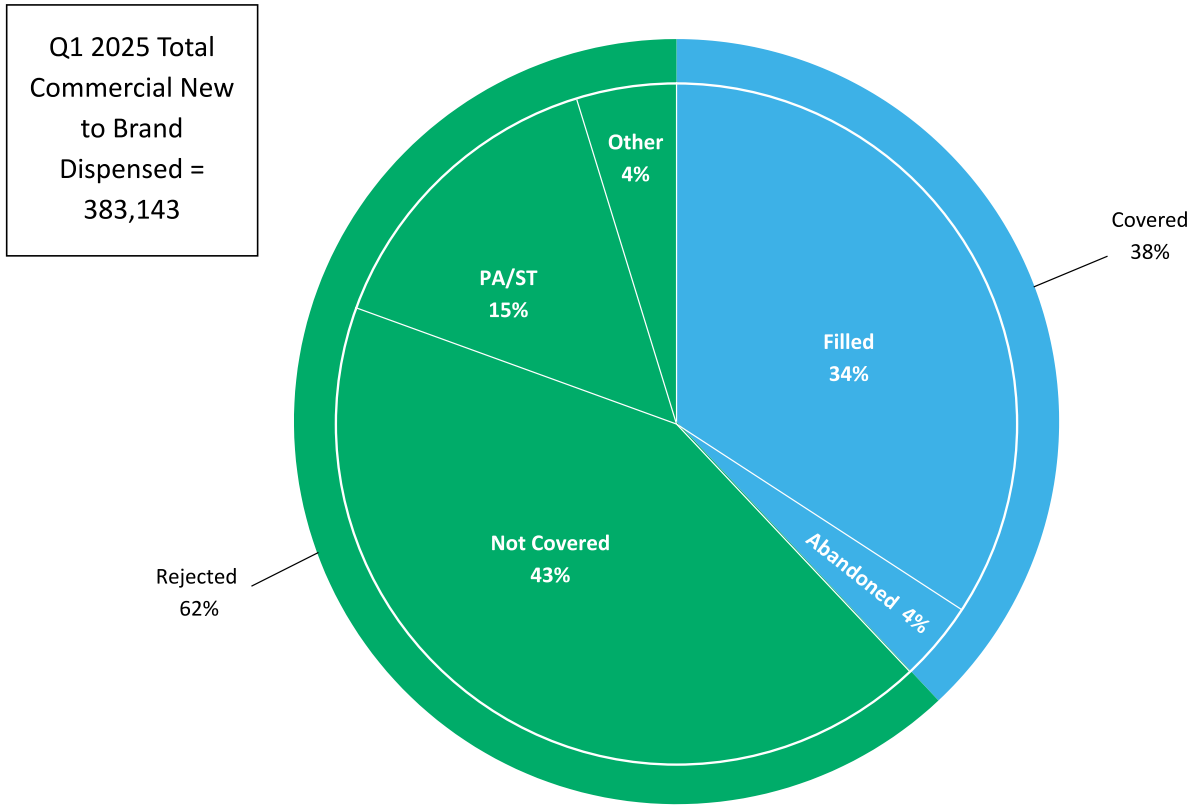
5.1. Coverage Policy Availability

Thirteen (72%) of the 18 large US commercial health plans in the SPEC database covered Zepbound, while the remaining five payers (28%) had no policy available as of the data cut-off date of April 2025. The absence of a policy does not necessarily imply payer non-coverage or plan exclusion, although both are possible.

5.2. Prior Authorization Burden

Based on data from IQVIA's LAAD database, over 3.5 million prescriptions of Zepbound were dispensed in the first quarter of 2025, of which about 10% (383,143) were new-to-brand prescriptions. Of the new-to-brand prescriptions, 38% were covered, of which 34% were filled, and 4% were abandoned. The remaining 62% were rejected due to prior authorization/step therapy reasons (15%), insurance non-coverage (43%), and other reasons, such as plan/refill limits, drug shortages, or administrative errors (4%). (Figure 5.1)

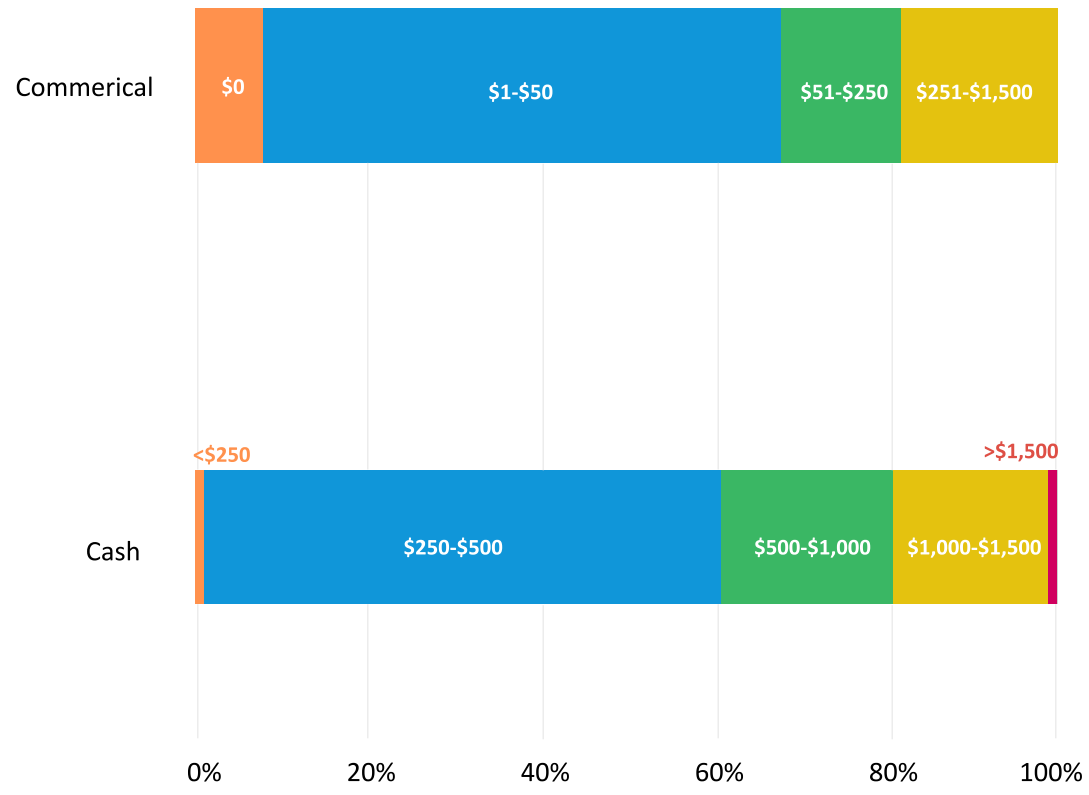
Figure 5.1. Prescription Outcomes of New-to-Brand Zepbound Prescriptions in Q1 2025



5.3. Patient Cost Sharing

In terms of out-of-pocket costs, the proportion of patients paying $0 was significantly smaller compared to the newly approved drugs presented above. For patients who had their prescription filled, over 60% paid less than $50, with the majority paying between $20 and $30. There was a significant proportion of new-to-brand cash prescriptions in Q1 2025 (136,939), approximately a 1:3 ratio, compared to the other written prescriptions. (See Figure 5.2) For those paying cash, the majority of patients paid $250-$500, likely reflecting discounts that matched the manufacturer's direct-to-consumer cash price of $349-$499, depending on the dose. However, 39% of patients also paid more than $500 for the prescription.

Figure 5.2. Patient Out-of-Pocket Costs for Zepbound, for Commercially Insured and Cash Pay Prescriptions, Q1 20225



5.4. Redirecting Excess Spending for Additional Access

The data above showed that the majority of prescriptions for Zepbound were rejected due to non-coverage. Given that ICER's recent review judged Zepbound to be high value from a long-term value perspective,[46] and the concern around short-term affordability, we conducted an exploratory analysis to show additional number of patients who could get access to Zepbound if the first-year excess spending ($1,264M ~ $1,494M) reported in Section 3 were redirected to cover Zepbound for patients who were denied access due to insurance non-coverage. Our analysis showed that at least 22%–26% of prescriptions rejected due to non-coverage in a year would be covered if the first year's excess spending were redirected, which would be a considerable improvement in access.

Table 5.1. Additional Drug Access from Redirected Excess Drug Spending

Drug Name	Current Access*			Additional Access
	Number of Commercial New-to-Brand Prescriptions Dispensed (Annualized)	Proportion of Prescriptions Rejected due to Insurance Non-Coverage	Number of New-to-Brand Prescriptions Not Covered	Additional Number of Individuals Gaining Access per Year
Zepbound	1,532,572	43%	659,006	145,079 ~ 171,460†

*Number of prescriptions was annualized based on Q1 2025 data from the IQVIA Longitudinal Access and Adjudication Data. Data includes commercial payers only.

†Based on the estimated annual net price of $8,714.86 for Zepbound.

5.5. Discussion

For GLP-1 drugs such as Zepbound, given their broad (and still expanding) clinical benefits, the population eligible for treatment is large and continues to grow. Although Zepbound is cost-effective at its current price, our data indicate that the majority of patients lack access to Zepbound, and those who do face high costs. In the first quarter of 2025, only about one-third of new-to-brand prescriptions were filled, likely due to the lack of coverage and coverage restrictions. It is likely that the large eligible population and high price of obesity medications make it difficult for payers to cover GLP-1s without substantial premium increases. One study found that a 1% rise in GLP-1 use for weight loss would result in an increase in 5% in the drug spend budget, equating to a $14.50 per member per month cost for employers.[47] It is no surprise, then, that more payers are dropping coverage, thus we may continue to see the majority of GLP-1 prescriptions be rejected. This raises questions about how best to structure coverage to maintain access and affordability for high-value drugs such as GLP-1s, which have a large eligible population. Data such as that abstracted from the SPEC and IQVIA databases can be used to benchmark whether the current benefit design achieves those goals. Finally, redirecting excess spending from areas where prices do not align with the added value they provide for patients could narrow the access gap, as our exploratory analysis showed.

6. Conclusion

In this report, we evaluated the year-to-year trend in launch prices, evaluated the health system impact of not aligning launch prices with overall health benefits, and assessed patient access barriers to newly launched drugs. Our analyses, as presented in Section 3, show that the inflation-adjusted launch prices of drugs (both list and net) continue to rise at a rate that exceeds inflation, GDP growth, and overall health care cost growth. This finding contributes to a more complete understanding of whether net prices are in fact dropping, as separating out the net pricing for recently-launched drugs versus those that have been on the market for many years may inform future policy action. Approximately 70% of the 23 ICER-reviewed drugs approved between 2022 and 2024 had launch net prices above what ICER had determined to be a fair price, based on the evidence at the time of launch. Aligning the prices of these therapies with ICER's HBPB could have saved the US health care system approximately $1.3 to $1.5 billion in the first year post-approval alone. In Section 3, we also presented the opportunity cost of not adopting value-based pricing, through equal value life years (evLYs) lost, health insurance coverage loss, and associated mortality. As illustrative examples, we calculated the number of additional people who could gain access to high-value drugs if the first-year excess spending on drugs priced above value were redirected. The Zepbound example in Section 5 demonstrated that redirecting first-year excess spending from drugs priced above ICER's HBPB could cover nearly one-quarter of rejected prescriptions for Zepbound. While these examples may be simplistic and do not account for the myriad factors that impede access, they highlight the significant opportunity to bridge the access gap for patients through value-based pricing. Furthermore, our evaluation of patient access in Section 4, although limited, underscores various barriers, including those related to insurance and non-insurance issues. The gaps in coverage policy availability particularly emphasize the challenges associated with new-to-market drugs and patient access.

Future research is needed to address some of the key limitations we encountered in developing this report:

1) There is a lack of transparency surrounding net prices. Net prices reflect discounts that encompass all concessions made by manufacturers but do not distinguish between the various types of concessions (e.g., rebates, co-payment cards, 340B discounts, etc.). Understanding the relationship between publicly available pricing data and overall net price estimates will be useful for future analyses. Although various policies have been implemented in recent years to enhance net price transparency, for these policies to be effective, strong enforcement, standardized reporting, and support from all stakeholders in the distribution system are essential.[48]

2) Our evaluation of patient access was limited to commercial payers. Future research should examine the prevalence of coverage for newly launched drugs based on payer type and explore the coverage restrictions across different market segments (including Medicare, Medicaid, self-insured, and fully insured plans).

Despite the limitations we have noted, we hope this report contributes to the critical decisions facing the US health care system and stimulates further bipartisan discussions on policy approaches to improve drug affordability and patient access. Finally, we would like to acknowledge and thank all our data partners, the patient representatives who shared their access experiences with us, as well as the individuals who provided ongoing input as part of our Working Group for this assessment. None of these organizations or individuals should be viewed as agreeing with our findings, and any errors in this paper are solely the responsibility of the authors.

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APPENDIX

A. Launch Price: Detailed Methodology

A1. Trend Analysis

A1.1. Data Sources

Drug Characteristics

We abstracted drug characteristics for each drug included in this report from the FDA's New Drug Therapy Approvals reports for drugs approved by CDER. Information was sourced manually from official review documents in Drugs@FDA for products regulated by CBER. Drug characteristics of interest included approval pathway (e.g., accelerated approval), special designations awarded (e.g., orphan product [proxy for population size, n<200,000], breakthrough), therapeutic area (e.g., oncology, cardiovascular, hematology), drug type (biologic vs. small molecule), gene/cell therapy, first-in-class mechanism, and first approved in the US. We examined the proportion of various drug characteristics across the three years. See Table A1.1.

Table A1.1. Drug Characteristics for Each Year (2022, 2023, 2024)

Drug Characteristics		2022 (n=39)	2023 (n=58)	2024 (n=52)
Indication*	Oncology	33%	26%	31%
	Dermatology	15%	7%	10%
	Endocrine/Metabolic	15%	14%	12%
	Hematology	10%	9%	12%
Drug Type	Biologic	51%	41%	44%
	Non-biologic	8%	10%	6%
	Small Molecule	41%	48%	50%
Type of Therapy	Gene/Cell Therapy	13%	10%	12%
Mechanism	First-in-Class	62%	43%	56%
Population Size	Orphan Product	62%	59%	63%
FDA Designation	Breakthrough	46%	19%	38%
Approval Pathway	Accelerated	18%	17%	19%
First Approved in US	First in US	67%	64%	65%

FDA: US Food and Drug Administration, n: number, US: United States

*Therapeutic areas that had at least six drugs in the category and were significantly associated with price were included in the analysis.

We abstracted characteristics of the pivotal trials that contributed to the FDA approval for each drug included in this report. Data included the number and Phase of clinical trials that were part of the FDA submission, type of clinical trial(s) (e.g., randomized trial, single-arm trial, etc.), and type of analysis (e.g., superiority, non-inferiority). We collected data from the FDA patient snapshot, FDA label, clinicaltrials.gov, and published manuscripts. If there was conflicting information, we prioritized information described in the FDA patient snapshot. One reviewer independently abstracted the data, and one reviewer validated the data.

List Price

For all drugs in scope, we obtained the list price or Wholesale Acquisition Cost (WAC) from Redbook at the time of launch (or closest to the time of launch). We used the unit WAC from Redbook and calculated the annual WAC based on dosing information from the FDA label. For drugs with multiple doses, we used the median dose. For weight-based dosing, we used a placeholder body weight based on the indicated population when available, or data from the clinical trial population. For drugs with dosing based on other variables (e.g., body surface area, number of chemotherapy cycles), we used clinical trial data to inform placeholder inputs for the dosing calculations. Table A1.2 lists the assumptions and placeholder inputs we used for annual calculations. For drugs that did not have WAC data available in Redbook, we relied on public manufacturer data when available, or other public sources of WAC data, including the California Health and Human Services Open Data Portal and the Oklahoma Health Care Authority Drug Utilization Review Board.

Table A1.2. Assumptions and Inputs Used in Annual Price Calculations

Drug	Assumptions and Inputs
Agamree	We used a placeholder body weight of 30 kg.
Alhemo	We used a placeholder body weight of 70 kg.
Alyftrek	We used the dosing regimen for patients aged 12 years and older.
Aphexda	We included only one dose in our calculation, assuming only a minority of patients use the optional second dose.
Aqneursa	We used the dosing regimen for patients weighing 35 kg or more.
Crenessity	We used the dosing regimen for the adult population.
Daxxify	We included two treatment sessions within our annual cost estimate.
Daybue	We used the dosing regimen for patients weighing 20 kg to 35 kg.
Defencath	We used a placeholder frequency of hemodialysis sessions of 3 times per week.
Duvyzat	We used a median of the weight-based dosing regimens included in the FDA label.
Elahere	We used a placeholder body weight of 60 kg.
Elfabrio	We used a placeholder body weight of 80 kg.
Enjaymo	We used a placeholder body weight of 80 kg.
Filsuvez	We assumed a month's supply is 27 tubes.

Drug	Assumptions and Inputs
Imjudo	We used a placeholder body weight of 80 kg.
Izervay	We assumed both eyes were treated in our calculations.
Lamzede	We used a placeholder body weight of 70 kg.
Lantidra	We assumed one infusion bag would be used.
Leqembi	We used a placeholder body weight of 80 kg.
Letybo	We included 3 treatments in our annual estimate.
Miebo	We assumed one package is one month's supply.
Miplyffa	We used the dosing regimen for patients weighing 30 kg to 55 kg.
Nemluvio	We used the dosing regimen for patients weighing less than 90 kg.
NexoBrid	We included one package in our annual cost.
Ngenla	We used a placeholder body weight of 35 kg.
Niktimvo	We used a placeholder body weight of 70 kg.
Ojemda	We used the 375 mg weekly dose in our calculations.
Pombiliti	We used a placeholder bodyweight of 70 kg.
Rapiblyk	We used a placeholder bodyweight of 70 kg, and calculated the median of the minimum and maximum dosages listed in the FDA label.
Revuforj	We used the dosing regimen for patients weighing 40 kg or more.
Rezdiffra	We used the dosing regimen for patients weighing less than 100 kg.
Rivfloza	We used the dosing regimen for patients weighing 50 kg or more.
Roctavian	We used a placeholder body weight of 70 kg.
Rolvedon	We assumed the chemotherapy cycle to be 28 days, for a total of 13 cycles per year.
Rystiggo	We used a placeholder of 4 cycles per year.
Rytelo	We used a placeholder body weight of 70 kg.
Ryzneuta	We used a placeholder of 4 cycles per year.
Sofdra	We estimated annual cost based on one pump per day.
Sohonos	We used the dosing regimen for patients 14 years and older.
Talvey	We used a placeholder body weight of 70 kg.
Veopoz	We used a placeholder body weight of 25 kg.
Voquezna Triple Pak	We included one kit in our calculation of annual cost.
Vtama	We assumed one tube is one month's supply.
Xenpozyme	We used a placeholder body weight of 50 kg.
Xolremdi	We used the dosing regimen for patients weighing 50 kg or more.
Ziihera	We used a placeholder weight of 70 kg.
Ztalmy	We used the dosing regimen for patients weighing more than 28 kg.

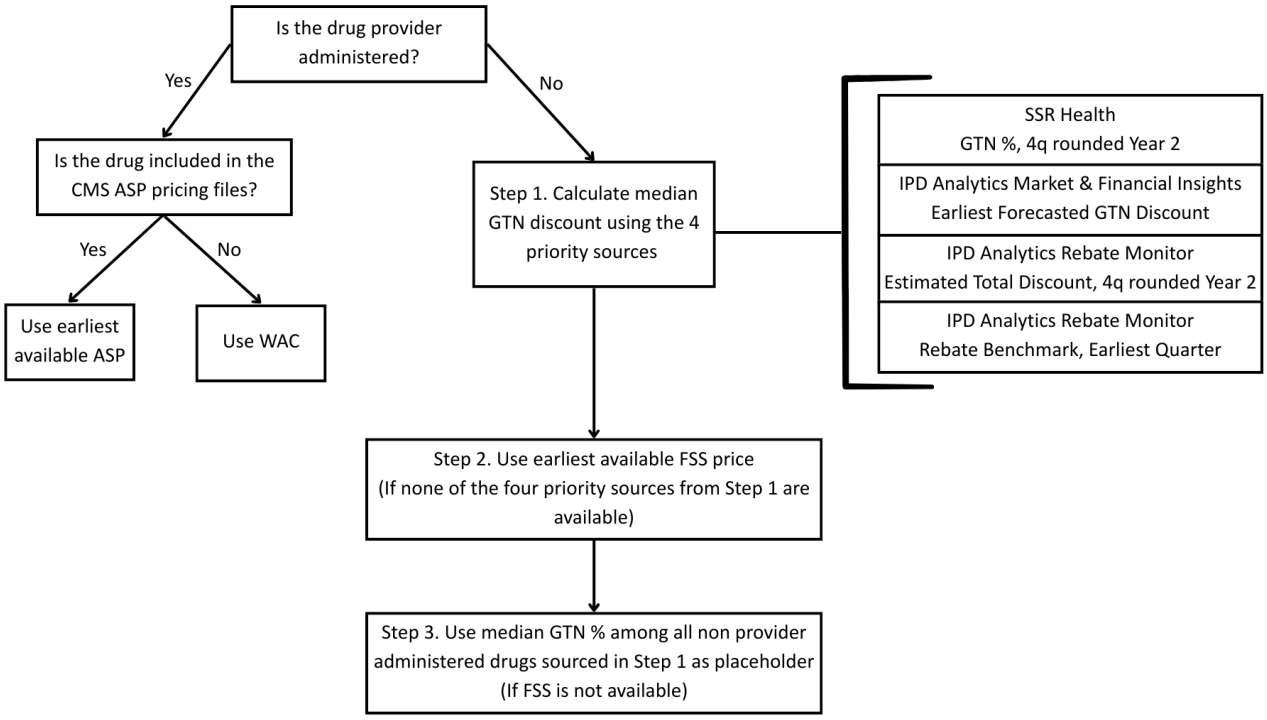
kg: kilogram

Net Price

We used multiple data sources to estimate the net price at the time of launch for drugs in scope (see Figure A1.1). We developed two separate approaches for provider-administered versus non-provider-administered drugs. For provider-administered drugs, we used ASP to represent net price. We sourced ASP from the CMS pricing files and removed the 6% markup included in the CMS payment allowance. For provider-administered drugs in scope that did not have pricing data available from CMS, we used WAC as a placeholder for net price. This approach was informed by the CMS reimbursement policy, which states that CMS reimburses Part B (provider-administered) drugs at the WAC price plus a 3% markup in the quarters before ASP becomes available.[49]

For non-provider-administered drugs, we used gross-to-net discount rates from multiple sources to calculate the net price. We prioritized data from the Rebate Benchmark and Estimated Total Discounts from the IPD Analytics Rebate Monitor, forecasted discounts from the IPD Analytics Market & Financial Insights, and gross-to-net discount rates from SSR Health. When more than one of these sources had data for a drug in scope, we calculated the median of all discount data available. If data was not available from any of these sources for a drug in scope, we used the FSS price closest to the time of launch. If FSS data were also not available, we used a placeholder gross-to-net discount rate calculated as the median discount among all other drugs in scope that were not provider-administered, which was 20%.

Figure A1.1. Prioritization of Net Price Data Sources Flowchart



ASP: average sales price, CMS: Centers for Medicaid and Medicare Services, FSS: federal supply schedule, GTN: gross-to-net, q: quarter, WAC: wholesale acquisition cost

For drugs in our scope that had previously been reviewed by ICER, we sent our list and net price estimates to manufacturers as part of our data request. Manufacturer data submissions were prioritized over other data sources for list and net price estimates.

A1.2. Data Analysis

List and Net Price

As noted in the main report, the list and net price data were heavily skewed due to some extremely expensive launch prices; therefore, we report the median prices in the unadjusted analysis. The list and net prices for drugs approved in 2022 and 2023 were inflation-adjusted to 2024 values.[23]

As also noted in the main report, we conducted trend analyses to examine changes in list and net prices at launch from 2022-2024, while controlling for drug characteristics listed above in Table A1.1. This is the adjusted analysis.

Therapeutic area (e.g., oncology, cardiovascular, hematology, etc.) was one of the drug characteristics of interest. To ensure robust adjustment for therapeutic area as a covariate, we only included therapeutic areas that had at least six drugs and were significantly associated with list or net price. We examined the median list and net price for each therapeutic area. See Table A1.3. We examined the association between therapeutic areas and list and net price using correlation analyses. See Table A1.4. Endocrine/metabolic drugs, hematology drugs, and oncology drugs were associated with significantly higher list and net price. Dermatology drugs had significantly lower list and net prices. We included these significant therapeutic areas as covariates in the trend analysis.

Table A1.3. Median List and Net Price Across Therapeutic Areas

Therapeutic Area*	Number of Drugs in Our Database	Median List Price (Range)	Median Net Price (Range)
Cardiovascular	7	$29,500 ($7,469-$245,140)	$29,500 ($3,245-$196,112)
Dermatology	15	$55,120 ($660-$1,294,502)	$35,828 ($593-$1,268,612)
Endocrine/Metabolic	20	$499,607 ($487-$4,250,000)	$473,653 ($390-$4,250,000)
Hematology	15	$795,600 ($11,721-$3,677,128)	676,260 ($8,598-$3,500,000)
Immunology	6	$529,223 ($18,889-$1,552,000)	$497,399 ($18,889-$1,552,000)
Neurology/CNS	15	$219,121 ($5,841-$3,285,018)	$220,217 ($2,191-$3,285,018)
Oncology	44	$358,461 ($12,114-$1,359,949)	$332,044 ($12,114-$1,359,949)

CNS: central nervous system

*Table only includes therapeutic areas with six or more drugs in our database.

Table A1.4. Therapeutic Areas Associated with List and Net Price

Therapeutic Area	Number of Drugs in Our Database	List Price Estimate	P-value	Net Price Estimate	P-value
Cardiovascular	7	-1.39	0.07	-1.54	0.06
Dermatology	15	**-1.70**	**0.001**	**-1.89**	**<0.001**
Endocrine/metabolic	20	**1.09**	**0.02**	**1.16**	**0.02**
Hematology	15	**1.35**	**0.01**	**1.40**	**0.01**
Immunology	6	0.99	0.24	1.14	0.19
Neurology/CNS	15	0.11	0.84	0.01	0.98
Oncology	44	**1.12**	**0.002**	**1.24**	**<0.001**

CNS: central nervous system

Therapeutic areas significantly associated with list and net price denoted in bold.

We conducted ordinary least squares regression analyses with log-transformed list or net price as the dependent variable and year approved as the independent variable. We included drug characteristics as covariates in the model. Due to a heavy skew, we log-transformed list and net price (outcome variable). We also conducted the regression analysis with log-transformed list or net price as the dependent variable, year approved as the independent variable, and drug characteristics and their interaction with year (the effect of one variable on the dependent variable depends on the level of another variable, e.g., year approved impacts list or net price only when the

population is rare) as covariates in the model. In these models, we were able to identify drug characteristics that are associated with list or net price as well as those that affect changes in list price over time.

All categorical covariates were converted into dummy variables for inclusion in the regression analyses. Drug type has three levels: biologic, non-biologic, and small molecule. As independent variables may be correlated (multicollinearity), we assessed the correlation between the independent variables in the model. For continuous variables, we examined the correlation coefficients; for categorical variables, we examined chi-square tests of independence for each pair of variables. We also examined Variance Inflation Factors (VIFs) post-model estimation. We removed the highly correlated variables to avoid multicollinearity. All analyses were conducted in R.

A1.3. Additional Results

We report a summary of the key findings in the main report. In this Appendix, we report the detailed multiple regression analyses, including the association between drug characteristics and list and net price.

Multiple Regression Results

List Price

We fitted a multiple regression model with year predicting the list price (log-transformed list price) while controlling for all drug characteristics. List prices increased over the three years, independent of drug characteristics, but the increase was not statistically significant (β=0.23, p=0.12). In this model, 57% of the variance was explained by the predictors (p<0.001). Oncology drugs, endocrine/metabolic drugs, gene/cell therapy, and orphan products were significant covariates and associated with higher list prices. There was no evidence of multicollinearity. See Table A1.5 for the percentage change in list price by each drug characteristic. See Table A1.7 for Beta estimates of the drug characteristics.

Next, we fitted a multiple regression model with year predicting list price (log-transformed list price) while controlling for all drug characteristics and their interaction with approval year. After controlling for drug characteristics and their interaction with year, list price increased each year, but the increase was not statistically significant (β=0.54, p=0.31). In this model, 58% of the variance was explained by year and the covariates (p<0.001). In this model, there were no significant predictors or interaction terms. See Table A1.8 for Beta estimates of the drug characteristics and their interactions.

Table A1.5. Multiple Log-Linear Regression Reporting Change in List Price by Year and Drug Characteristics

Drug Characteristics		List Price			
		Median List Price Annual Price (Range)	Relative Change (Unadjusted)	Percentage Change (Unadjusted)	Percentage Change (Adjusted)*
All Drugs (N=149)		$285,808 ($487-$4,250,000)	Reference	Reference	Reference
Year†	**2022, n=39**	$249,257 ($853-$3,677,128)	Reference	Reference	Reference
	2023, n=58	$306,937 ($487-$3,285,018)	+$59,492	+24%	+25% per year (95% CI: -6 to 67%; p=0.12)
	2024, n=52	$308,749 ($660-$4,250,000)			
Indication‡	**Oncology, n=44**	$358,461 ($12,114-$1,359,949)	+$205,088	+134%	**+162% (95% CI: 43 to 381%; p=0.002)**
	Dermatology, n=15	$55,120 ($660-$1,294,502)	-$258,570	-82%	-14% (95% CI: -63 to 98%; p=0.72)
	Endocrine or Metabolic, n=20	$499,607 ($4,487-$4,250,000)	+$247,461	+98%	**+140% (95% CI: 10 to 426%; p=0.03)**
	Hematology, n=15	$795,600 ($11,721-$3,677,128)	+$536,949	+208%	+114% (95% CI: -14 to 437%; p=0.10)
Drug Type	**Biologic, n=67**	$380,981 ($508-$4,250,000)	Reference	Reference	Reference
	Non-Biologic, n=12	$285,808 ($12,114-$774,607)	-$95,173	-25%	-24% (95% CI: -70 to 92%; p=0.56)
	Small Molecule, n=70	$165,717 ($487-$967,433)	-$215,264	-57%	-16% (95% CI: -51 to 43%; p=0.51)
Type of Therapy	**Gene/Cell Therapy, n=17**	$2,511,884 ($252,146-4,250,000)	+$2,267,045	+926%	**+320% (95% CI: 85 to 853%; p=0.001)**
	Not gene/Cell Therapy, n=132	$244,839 ($487-$1,552,000)	Reference	Reference	Reference

Drug Characteristics		List Price			
		Median List Price Annual Price (Range)	Relative Change (Unadjusted)	Percentage Change (Unadjusted)	Percentage Change (Adjusted)*
Mechanism	**First-in-Class, n=78**	$377,524 ($853-$3,500,000)	+$217341	+136%	+48% (95% CI: -9 to 141%; p=0.11)
	Not First-in-Class, n=71	$160,183 ($487-$3,500,000)	Reference	Reference	Reference
Population Size	**Orphan Product, n=91**	$409,169 ($3,309-$4,250,000)	+$371,452	+985%	**+551% (95% CI: 286 to 997%; p<0.001)**
	Not Orphan Product, n=58	$37,717 ($487-$1,294,502)	Reference	Reference	Reference
FDA Designation	**Breakthrough, n=49**	$362,212 ($27,204-$3,677,128)	+141947	+64%	+26% (95% CI: -26 to 116%; p=0.39)
	Not Breakthrough, n=100	$220,265 ($487-$4,250,000)	Reference	Reference	Reference
Approval Pathway	**Accelerated, n=27**	$359,298 ($27,204-$3,950,000)	+$105,718	+42%	0% (95% CI: -48 to 93%; p=0.99)
	Not Accelerated, n=122	$253,580 ($487-$4,250,000)	Reference	Reference	Reference
First Approved in US	**First in US, n=97**	$305,903 ($487-$3,677,128)	+$127,716	+72%	-6% (95% CI: -44 to 58%; p=0.82)
	Not First in US, n=52	$178,187 ($508-$4,250,000)	Reference	Reference	Reference

CI: confidence interval, n: number, US: United States

Bold signifies significant independent variables in the model.

*We exponentiated log-transformed list price to provide percentage change

†Year was included as a linear factor in the adjusted analysis

‡All therapeutic areas that had at least six drugs in the category and significantly associated with price were included in the multiple regression

§Each indication is compared to all other drugs for relative and percentage change, e.g., oncology vs. non-oncology drugs

Net Price

We fitted a multiple regression model with year predicting net price (log-transformed net price) while controlling for all drug characteristics. Net prices increased over the three years (β=0.29, p=0.054), independent of drug characteristics. In this model, 59% of the variance was explained by the predictors (p<0.001). Oncology drugs, endocrine/metabolic drugs, gene/cell therapy, and orphan products were significant covariates and associated with higher net prices. There was no evidence of multicollinearity. See Table A1.6 for the percentage change in list price by each drug characteristic. See Table A1.7 for Beta estimates of the drug characteristics.

Next, we fitted a multiple regression model with year predicting net price (log-transformed net price) while controlling for all drug characteristics and their interaction with approval year. After controlling for drug characteristics and their interaction with year, net price increased each year, but the increase was not statistically significant (β=0.56, p=0.28). In this model, 60% of the variance was explained by year and the covariates (p<0.001). In this model, there were no significant predictors or interaction terms. See Table A1.8 for Beta estimates of the drug characteristics and their interactions.

Table A1.6. Multiple Log-Linear Regression Reporting Change in Net Price by Year and Drug Characteristics

Drug Characteristics		Net Price			
		Median Net Price Annual Price	Relative Change (Unadjusted)	Percentage Change (Unadjusted)	Percentage Change (Adjusted)*
All Drugs (N=149)		$242,937 ($390-$4,250,000)	Reference	Reference	Reference
Year†	**2022, n=39**	$182,271 ($452-$3,151,824)	Reference	Reference	Reference
	2023, n=58	$264,938 ($390-$3,285,018)	+$71,104	+39%	+32% per year (95% CI: -1 to 79%; p=0.054)
	2024, n=52	$274,795 ($660-$4,250,000)			

Drug Characteristics		Net Price			
		Median Net Price Annual Price	Relative Change (Unadjusted)	Percentage Change (Unadjusted)	Percentage Change (Adjusted)*
Indication‡§	Oncology, n=44	$332,044 ($12,114-$1,359,949)	+$206,557	+165%	**+174% (95% CI: 47 to 409%; p=0.002)**
	Dermatology, n=15	$35,828 ($593-$1,268,612)	-$243,009	-87%	-22% (95% CI: -67 to 83%; p=0.56)
	Endocrine or Metabolic, n=20	$473,653 ($390-$4,250,000)	+$277,541	+142%	**+141% (95% CI: 8 to 436%; p=0.03)**
	Hematology, n=15	$676,260 ($8,598-$3,500,000)	+$461,027	+214%	+108% (95% CI: -19 to 433%; p=0.13)
Drug Type	Biologic, n=67	$375,880 ($508-$4,250,000)	Reference	Reference	Reference
	Non-Biologic, n=12	$286,166 ($4,814-$762,930)	-$89,714	-24%	-29% (95% CI: -72 to 84%, p=0.48)
	Small Molecule, n=70	$137,518 ($390-$919,061)	-$238,362	-63%	-31% (95% CI: -60 to 19%; p=0.18)
Type of Therapy	Gene/Cell Therapy, n=17	$2,258,450 ($251,771-$4,250,000)	+2,070,449	+1101%	**+327% (95% CI: 85 to 887; p=0.001)**
	Not Gene/Cell Therapy, n=132	$188,001 ($390-$1,553,935)	Reference	Reference	Reference
Mechanism	First-in-Class, n=78	$365,015 ($452-$4,250,000)	+$226,979	+164%	+41% (95% CI: -14 to 132%; p=0.17)
	Not First-in-Class, n=71	$138,036 ($390-$3,500,000)	Reference	Reference	Reference

Drug Characteristics		Net Price			
		Median Net Price Annual Price	Relative Change (Unadjusted)	Percentage Change (Unadjusted)	Percentage Change (Adjusted)*
Population Size	**Orphan Product, n=91**	$395,210 ($3309-$4,250,000)	+360,297	+1032%	**+651% (95% CI: 340 to 1182%; p<0.001)**
	Not Orphan Product, n=58	$34,913 ($390-$1,268,612)	Reference	Reference	Reference
FDA Designation	**Breakthrough, n=49**	$357,623 ($27,194-$3,500,000)	+$170,171	+91%	+33% (95% CI: -23 to 131%; p=0.31)
	Not Breakthrough, n=100	$187,452 ($390-$4,250,000)	Reference	Reference	Reference
Approval Pathway	**Accelerated, n=27**	$359,286 ($27,194-$3,950,000)	+$159,224	+80%	0% (95% CI: -49 to 96%; p=0.99)
	Not Accelerated, n=122	$200,062 ($390-4,250,000)	Reference	Reference	Reference
First Approved in US	**First in US, n=97**	$267,905 ($390-$3,285,018)	+$87,313	+48%	-4% (95% CI: -44 to 63; p=0.87)
	Not First in US, n=52	$180,592 ($452-$4,250,000)	Reference	Reference	Reference

CI: confidence interval, n: number

All analyses were conducted in R.

*We exponentiated log-transformed list price to provide percentage change

†Year was included as a linear factor in the adjusted analysis

‡All therapeutic areas that had at least six drugs in the category and significantly associated with price were included in the multiple regression

§Each indication is compared to all other drugs for relative and percentage change, e.g., oncology versus non-oncology drugs

Table A1.7. Multiple Log-Linear Regression Predictors for List and Net Price

Predictor/Covariate	List Price Estimate	p-Value	Net Price Estimate	p-Value	VIF
Intercept (Baseline Price)	9.53	<0.001	9.23	<0.001	-
Year	0.23	0.12	0.29	0.054	1.02
Oncology	0.97	0.002	1.01	0.002	1.55
Dermatology	-0.15	0.72	-0.25	0.56	1.28
Endocrine or Metabolic	0.88	0.03	0.88	0.03	1.44
Hematology	0.76	0.10	0.73	0.13	1.55
Drug type: Biologic vs. Non-Biologic	-0.28	0.56	-0.34	0.48	1.51
Drug Type: Biologic vs. Small Molecule	-0.18	0.51	-0.48	0.18	
Gene or Cell Therapy	1.44	<0.001	1.45	0.001	1.37
First-in-Class	0.39	0.11	0.34	0.17	1.20
Orphan Product	1.87	<0.001	2.02	<0.001	1.32
Breakthrough Designation	0.23	0.39	0.29	0.31	1.30
Accelerated Approval	-0.002	0.99	-0.005	0.99	1.31
First Approved in US	-0.06	0.82	-0.04	0.87	1.23

US: United States, VIF: Variance Inflation Factor

Table A1.8. Multiple Log-Linear Regression Predictors (and Interactions) for List and Net Price

Predictor/Covariate	List Price Estimate	p-Value	Net Price Estimate	p-Value	VIF†
Intercept (Baseline price)	8.82	<0.001	8.57	<0.001	-
Year	0.54	0.31	0.59	0.28	12.55
Oncology	1.21	0.24	1.25	0.24	16.51
Dermatology	-0.24	0.84	-0.42	0.72	9.12
Endocrine or Metabolic	1.44	0.24	1.33	0.29	12.82
Hematology	2.09	0.15	1.85	0.21	13.76
Drug Type: Biologic vs. Non-Biologic	-0.15	0.92	-0.50	0.75	127.12
Drug Type: Biologic vs. Small Molecule	0.29	0.72	-0.13	0.87	
Gene or Cell Therapy	1.76	0.15	1.64	0.19	11.02
First-in-Class	0.75	0.34	0.69	0.39	11.29
Orphan Product	1.18	0.14	1.59	0.054	11.25
Breakthrough Designation	0.42	0.61	0.61	0.48	11.19
Accelerated Approval	0.95	0.37	0.79	0.47	12.32
First Approved in US	0.13	0.87	0.08	0.92	10.32
Year * Oncology	-0.13	0.77	-0.13	0.78	16.60
Year * Dermatology	0.07	0.90	0.11	0.85	9.66
Year * Endocrine or Metabolic	-0.28	0.62	-0.22	0.71	13.29
Year * Hematology	-0.64	0.31	-0.54	0.41	14.26

Predictor/Covariate	List Price Estimate	p-Value	Net Price Estimate	p-Value	VIF†
Year * Drug Type: Biologic vs. Non-Biologic	-0.04	0.96	0.10	0.89	152.07
Year * Drug Type: Biologic vs. Small Molecule	-0.21	0.57	-0.11	0.78	
Year * Gene or Cell Therapy	-0.18	0.74	-0.12	0.83	11.01
Year * First-in-Class	-0.18	0.60	-0.17	0.63	13.04
Year * Orphan Product	0.35	0.34	0.22	0.56	14.65
Year * Breakthrough Designation	-0.09	0.80	-0.16	0.67	12.08
Year * Accelerated Approval	-0.42	0.37	-0.35	0.47	13.01
Year * First Approved in US	-0.08	0.83	-0.04	0.91	14.86

US: United States, VIF: Variance Inflation Factor

Denotes an interaction (e.g., year oncology = whether the effect of year changes for oncology versus non-oncology drugs).

†As these predictors include interaction terms, VIF was higher than what was deemed normal.

Sensitivity Analyses

While the primary analysis, which includes all covariates, provides a comprehensive assessment of their impact, a more parsimonious model can help identify the most relevant predictors and reduce potential overfitting. We conducted sensitivity analyses that only included the most relevant variables that predicted list price and assess the robustness of the primary analysis results.[50] We ran two regression models using a variable selection technique: stepwise regression analyses. In the two stepwise regression analyses, year was included as a predictor of list or net price as the null model, and all drug characteristics were added as predictors into the stepwise model.

List Price

We conducted a stepwise regression analysis with year and all drug characteristics predicting list price (log-transformed list price). List price increased over the three years (β=0.22, p=0.12), independent of orphan drugs, gene/cell therapy, first-in-class, oncology, endocrine/metabolic, and hematology drugs. The model accounts for 56% of the variance in list price (p<0.001). See Table A1.9 for Beta values. The sensitivity analysis results are consistent with the base-case multiple regression described above.

In a stepwise regression analysis with year, all drug characteristics, and drug characteristics and their interaction with year predicting list price (log-transformed list price), the model results were the same as the model containing year and drug characteristics alone.

Table A1.9. Stepwise Regression Predictors for List Price

Predictor/Covariate	List Price Estimate	p-Value
Intercept	9.34	<0.001
Year	0.22	0.12
Orphan Product	1.90	<0.001
Gene or Cell Therapy	1.53	<0.001
Oncology Drugs	1.09	<0.001
Endocrine/Metabolic Drugs	0.96	0.01
Hematology Drugs	0.92	0.03
First-in-Class	0.44	0.06

Net Price

Net prices increased over the three years (β=0.28, p=0.058), independent of significant covariates: orphan product, gene/cell therapy, oncology, endocrine/metabolic, hematology, and first-in-class. The model accounts for 58% of the variance in list price (p<0.001). See Table A1.10 for Beta values. The sensitivity analysis results are consistent with the base-case multiple regression described above.

In a stepwise regression analysis with year, all drug characteristics, and drug characteristics and their interaction with year predicting net price (log-transformed net price), the model results were the same as the model containing year and drug characteristics alone.

Table A1.10. Stepwise Regression Predictors for Net Price

Predictor/Covariate	List Price Estimate	p-Value
Intercept	8.90	<0.001
Year	0.28	0.058
Orphan Product	2.08	<0.001
Gene or Cell Therapy	1.63	<0.001
Oncology	1.19	<0.001
Endocrine/Metabolic	1.04	0.01
Hematology	0.95	0.03
First-in-Class Product	0.40	0.098

Quantile Regression Results

Because data were heavily skewed and median values may better represent the unadjusted data, we also conducted quantile regression analyses with year predicting list and net price, while controlling for drug characteristics. Quantile regression estimates the median of the response variable, compared to ordinary least squares regression which estimates the mean. We estimated quantile regressions of list and net price at the 25th, 50th, and 75th percentiles, while controlling for drug characteristics. Standard errors were bootstrapped with 500 repetitions.

List Price

At the median, year was associated with higher list prices (+$25,115 per year, p=0.33), independent of all drug characteristics. Endocrine/metabolic drugs, gene/cell therapies, and orphan products were associated with higher list prices at the median. At the 25th percentile, year was associated with higher list prices (+$9,469 per year, p=0.67). Only orphan product was significantly associated with higher list prices at this percentile. At the 75th percentile, year was associated with significantly higher list prices (+$68,315 per year, p=0.05), independent of all drug characteristics. Gene/cell therapies, first-in-class, and orphan products were associated with higher net prices at the 75th percentile. See Table A1.10.

Table A1.11. Predictors in the Quantile Regression of List Price

Predictor/Covariates	25th Percentile Coefficient (SE), p-Value	Median Coefficient (SE), p-Value	75th Percentile Coefficient (SE), p-Value
Year	9,469 (21791), p=0.67	25,115 (25836), p=0.33	**68,315 (34619), p=0.05**
Oncology Drugs	111,309 (77810), p=0.15	136,399 (78200), p=0.08	93,884 (70743), p=0.19
Dermatology Drugs	-6,750 (55490), p=0.90	2,018 (51730), p=0.097	-64,908 (69234), p=0.35
Endocrine/Metabolic Drugs	109,153 (122089), p=0.37	**338,977 (148980), p=0.02**	298,187 (184436), p=0.11
Hematology Drugs	70,422 (193411), p=0.72	281,946 (328618), p=0.39	29,496 (265367), p=0.91
Non-Biologic*	-29,656 (100161), p=0.77	-69,518 (109087), p=0.53	-201,196 (152497), p=0.19
Small Molecule*	-23,595 (44026), p=0.59	-19,319 (43727), p=0.66	-62,954 (79371), p=0.43
Gene/Cell Therapies	220,443 (575019), p=0.70	**1,927,524 (825739), p=0.02**	**2,857,508 (376725), p<0.001**
First-in-Class	32,585 (35092), p=0.35	19,987 (48309), p=0.68	**140,867 (66199), p=0.04**
Orphan Products	**166,677 (64480), p=0.01**	**205,412 (74028), p=0.01**	**324,175 (82243), p<0.001**
Breakthrough Designation	19,307 (52952), p=0.072	37,649 (68967), p=0.59	64,737 (86373), p=0.45
Accelerated Approval	-9,204 (70121), p=0.90	-52,760 (73531), p=0.47	-171,638 (119405), p=0.15
First in US	16,593 (40822), p=0.69	-1,980 (49340), p=0.97	-21,664 (70185), p=0.76

SE: standard error, US: United States

Bold signifies statistical significance.

*Biologic used as the reference group

Net Price

At the median, year was associated with higher net prices (+$30,787 per year, p=0.24), independent of all drug characteristics. Oncology and endocrine/metabolic drugs, gene/cell therapies, and orphan products were associated with higher net prices at the median. At the 25th percentile, year was associated with higher net prices (+$6,756 per year, p=0.75). Only orphan product was significantly associated with higher net prices at this percentile. At the 75th percentile, year was associated with significantly higher net prices (+$73,156 per year, p=0.03), independent of all drug characteristics. Gene/cell therapies, first-in-class drugs, and orphan products were associated with higher net prices at the 75th percentile. See Table A1.12.

Table A1.12. Predictors in the Quantile Regression of Net Price

Predictor/Covariates	25th Percentile Coefficient (SE), p-Value	Median Coefficient (SE), p-Value	75th Percentile Coefficient (SE), p-Value
Year	6,756 (20985), p=0.75	30,787 (25952), p=0.24	**73,156 (33178), p=0.03**
Oncology Drugs	84,189 (67043), p=0.21	**129,422 (57463), p=0.03**	1034,000 (70148), p=.014
Dermatology Drugs	-6,604 (49005), p=0.89	-27,901 (50762), p=0.58	-69,215 (65946), p=0.30
Endocrine/Metabolic Drugs	95,438 (113857), p=0.40	**368,416 (154611), p=0.02**	343,252 (198090), p=0.09
Hematology Drugs	60,284 (192715), p=0.75	266,170 (234563), p=0.23	6,931 (227616), p=0.98
Non-Biologic*	-11,417 (95451), p=0.90	-96,717 (100678), p=0.34	-201,125 (139344), p=0.15
Small Molecule*	-18,032 (42336), p=0.67	-54,668 (39544), p=0.17	-99,032 (77491), p=0.20
Gene/Cell Therapies	279,867 (560988), p=0.62	**1,736,243 (684225), p=0.01**	**2,747,316 (485565), p<0.001**
First-in-Class	20,207 (36563), p=0.58	46,270 (42559), p=0.28	**141,284 (63503), p=0.03**
Orphan Products	**140,234 (54744), p=0.01**	**183,072 (60924), p=0.03**	**285,150 (72342), p<0.001**
Breakthrough Designation	14,961 (46640), p=0.75	-25,851 (65859), p=0.70	32,287 (87158), p=0.071
Accelerated Approval	-1,720 (57010), =0.98	-16,314 (71485), p=0.82	-148,057 (118451), p=0.21
First in US	13,445 (39955), p=0.74	-12,530 (42648), p=0.77	-43,639 (59648), p=0.47

SE: standard error, US: United States

Bold signifies statistical significance.

*Biologic used as the reference group

Overall, results from both list and net price quantile regression show consistency across the median and percentiles, and these results are in alignment with the least squares regression analysis results reported in the main report. Year had the largest association with list and net price at the top 75th percentile, demonstrating that later years are associated with higher launch prices, particularly among the most expensive drugs.

A1.4. Exploratory Analysis

Clinical Trial Data

We obtained data from the pivotal trials of the drugs. On average, each drug in scope had 1.51 pivotal clinical trials included in the FDA approval package (median: 1), with an average of 673 participants (median: 202). The majority of drugs in scope had controlled trials within their FDA approval package, although over half (51%) included placebo-controlled trials, while only 28% included active-controlled trials. Most drugs included randomized trials in their package (73%), whereas 28% included single-arm trials.

We conducted exploratory analyses examining the association of clinical trial characteristics with list and net price. We examined the following clinical trials characteristics: number of studies, number of participants, whether the FDA package included single-arm trials, randomized trials, placebo-controlled trials, or active-controlled trials.

As reported above, our trend analysis showed that orphan products were associated with higher list and net prices. Orphan products are often evaluated in single-arm trials with small patient populations, given the rarity of the condition. This pattern also applies to many gene/cell therapies, which were also associated with higher list and net prices. Consistent with this, we found that drugs with higher list and net prices were more likely to be evaluated in single-arm trials, with fewer trials and smaller sample sizes.

Table A1.13. Table Key for Table A1.14

Key		
Number of Trials	1	
	2	
	3	
	4	
	≥5	
Trial Characteristics	Yes	
	No	

Table A1.14. Characteristics of Pivotal Trials of Drugs In Scope (Approved in 2022-2024)

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
2022 Approvals									
Skysona	$3,000,000.00		67	2/3 and 3					24 months
Hemgenix	$3,500,000.00		54	3					7-18 months post treatment
Zynteglo	$2,800,000.00		41	3					12 to 24 months post-transplant
Kimmtrak	$1,294,440.00		378	2					14.1 months median follow-up
Xenpozyme	$871,324.00		38	1/2, 2, 2/3					52 or 64 weeks and up to 9 years
Carvykti	$465,000.00		516	1/2 and 3					Up to 45.2 months or 3.9 years
Amvuttra	$463,500.00		122	3					9 months
Elahere	$431,253.25		106	3					Up to 15 months
Tecvayli	$369,930.00		110	2					Up to 2.9 years
Opdualag	$356,049.00		714	2/3					Up to 33 months
Lunsumio	$340,395.98		90	1/2				*	Up to 4 years
Enjaymo	$340,200.08		24	3					26 weeks
Rezlidhia	$391,766.69		153	1/2					Up to 30 weeks
Pyrukynd	$335,800.00		107	3					16, 20, or 24 weeks
Pluvicto	$255,000.00		831	3					Up to 32 months
Adstiladrin	$240,000.00		103	3					12 months
Lytgobi	$304,253.60		103	1/2				*	Up to 50.5 months
Ztalmy	$289,677.87		101	3					17 weeks
Vonjo	$237,250.00		63	3					24 weeks
Krazati	$240,291.62		112	3					Up to 143 weeks
Tzield	$193,900.00		76	2					745 days median

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Relyvrio	$163,001.70		137	2					24 weeks
Briumvi	$98,333.30		1093	3					Up to 96 weeks
Camzyos	$89,499.93		251	3					30 weeks
Terlivaz	$60,800.00		199	3					14 days
Rolvedon	$58,500.00		643	3					21 days
Spevigo	$51,133.00		53	2					1 week
Imjudo	$48,750.00		782	3					46 months
Sunlenca	$43,062.50		36	2/3					15 days
Sotyktu	$74,999.94		1684	3					16 weeks
Cibinqo	$59,787.00		1615	3					12 weeks
Vabysmo	$11,860.00		2591	3					48 or 56 weeks
Mounjaro	$12,666.29		6263	3					40 or 52 weeks
Vtama	$16,120.81		1025	3					12 weeks
Nexobrid	$3,150.00		331	3					2 hours
Quviviq	$5,560.05		1854	3					1 or 3 months
Vivjoa	$2,700.00		871	3					48 weeks
Daxxify	$840.00		609	3					4 weeks
Voquezna Triple Pak	$812.00		992	3					4 weeks
Omlonti	‡		1203	3					1 or 6 weeks and 3 months
2023 Approvals									
Elevidys	$3,200,000.00		214	1, 1/2, and 3					12, 48, or 52 weeks
Lyfgenia	$3,100,000.00		36	1/2					6 or 18 months

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Roctavian	$2,446,875.00		134	3					Up to 2 years
Casgevy	$2,200,000.00		44	2/3					16 months
Lamzede	$1,456,000.00		25	3					52 weeks
Vyjuvek	$1,261,000.00		31	3					26 weeks
Veopoz	$1,168,269.00		10	2/3					12 or 48 weeks
Rivfloza	$754,560.00		29	2					90 to 180 days
Pombiliti	$618,799.85		123	3					52 weeks
Sohonos	$624,150.00		213	3					Up to 24 months
Joenja	$547,500.00		31	2/3					85 days
Daybue	$616,120.00		187	3					12 weeks
Elrexfio	$490,432.32		187	2					Up to 16 months
Fabhalta	$550,000.03		137	3					14 to 168 days
Zilbrysq	$524,840.40		174	3					12 weeks
Elfabrio	$430,051.44		16	1/2					12 months
Filsuvez	$583,200.17		223	3					45 days
Wainua	$498,999.94		168	3					35 to 66 weeks
Vanflyta	$398,580.00		539	3					3 years
Epkinly	$396,170.11		148	1/2					Up to 1.5 years
Talvey	$371,121.00		187	1					Up to 28 days and 2.1 years
Columvi	$349,999.38		145	1					Up to 5 years
Omisirge	$338,000.00		125	3					42 days
Augtyro	$363,412.25		351	2					2 to 3 years
Lantidra	$300,000.00		30	1/2 and 3					12 or 15 months
Ojjaara	$327,283.35		376	3					24 weeks
Rystiggo	$290,400.00		200	3					43 days
Skyclarys	$375,138.90		103	2					12 or 48 weeks
Ogsiveo	$352,837.69		142	3					Up to 2 years

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Orserdu	$259,880.00		478	3					12 months
Qalsody	$213,450.01		108	3					28 weeks
Fruzaqla	$327,600.00		1107	3					Up to 22 months or 2 years
Jaypirca	$255,500.00		120	2					24 months
Zynyz	$185,120.00		65	2					Up to 26.8 months
Truqap	$297,986.04		289	3					Up to 51 months
Loqtorzi	$154,128.52		461	1/2 and 3					Up to 1.5 or 2 years
Agamree	$156,037.50		121	2					24 weeks
Omvoh	$150,299.00		1062	3					12 or 40 weeks
Filspari	$120,450.00		281	3					36 weeks
Ngenla	$99,699.60		224	3					52 weeks
Bimzelx	$66,387.60		839	3					16 weeks
Velsipity	$74,999.94		741	3					12 or 52 weeks
Izervay	$50,400.00		625	2/3 and 3					12 or 24 months
Defencath	$38,998.44		806	3					4 to 884 days
Litfulo	$49,134.62		718	2/3					24 weeks
Leqembi	$26,500.24		1795	3					18 months
Ryzneuta	$18,400.00		515	3					Average of 3 weeks
Aphexda	$11,800.00		122	3					6 days
Zurzuvae	$15,900.00		345	3					15 days
Jesduvroq	$11,417.20		2964	3					28 to 52 weeks and up to 3.9 person years
Zavzpret	$17,600.00		2870	3					2 hours
Rezzayo	$5,850.00		199	3					14 and 30 days
Veozah	$6,691.65		1022	3					4 or 12 weeks
Inpefa	$7,275.65		11806	3					Up to 21.9 months or 30 months

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Miebo	$9,252.00		1217	3					57 days
Xdemvy	$1,850.00		833	2/3 and 3					43 days
Beyfortus	$495.00		2943	2 and 3					150 days
Brenzavvy	$474.50		3346	3					24 to 60 weeks
Paxlovid	‡		3379	2/3					28 days
Exxua	‡		442	3					8 weeks
2024 Approvals									
Lenmeldy	$4,250,000.00		37	1/2 and 2					24 months
Kebilidi	$3,950,000.00		13	2					48 weeks
Beqvez	$3,500,000.00		45	3					15 months post infusion
Ryoncil	$1,552,000.00		54	3					28 days
Miplyffa	$967,432.50		50	2/3					12 months
Alhemo	$888,552.00		133	3					32 weeks
Revuforj	$810,984.37		104	1/2					Approximately 1 or 3 years
Tecelra	$727,000.00		44	2					Up to 2 years
Hympavzi	$795,600.00		116	3				†	18 months
Bizengri	$617,500.00		138	2					36 months
Duvyzat	$675,032.21		179	3					18 months
Tryngolza	$595,008.00		66	3					6 months
Aqneursa	$701,321.42		60	3					24 weeks
Ziihera	$554,580.00		80	2					Up to 34 months
Piasky	$551,839.55		204	3					25 weeks
Anktiva	$537,000.00		88	2/3					12 and 60 months
Aucatzyl	$525,000.00		65	1/2					Up to 24 months
Amtagvi	$515,000.00		153	2					Up to 60 months
Crenessity	$466,384.83		285	3					4 or 24 weeks
Xolremdi	$496,400.00		31	3					52 weeks

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Voranigo	$485,218.83		331	3					30 months
Imdelltra	$400,71.00		99	2					Up to 24 months
Rytelo	$354,780.69		178	2/3					Up to 5 years
Niktimvo	$319,410.00		79	2					24 weeks
Ojemda	$330,720.00		137	2					Up to 48 months
Alyftrek	$370,269.29		971	3					24 weeks
Itovebi	$298,087.68		325	2/3					Up to 3.7 years
Yorvipath	$285,808.04		82	3					26 weeks
Ensacove	$255,014.55		290	3					36 months
Winrevair	$245,140.00		323	3					24 weeks
Lazcluze	$221,409.00		858	3					Up to 32.8 months
Tevimbra	$180,405.33		512	3					10 months or 2 years
Attruby	$244,538.52		611	3					30 months
Vyloy	$175,968.00		1072	3					Up to 47 or 62 months
Livdelzi	$153,373.00		193	3					12 months
Iqirvo	$139,430.00		161	3					52 weeks
Leqselvi	$55,525.14		1209	3					24 weeks
Rezdiffra	$48,058.35		888	3					12 months
Ebglyss	$66,500.00		1062	3					16 weeks
Voydeya	$50,260.50		84	3					12 weeks
Nemluvio	$55,120.00		560	3					16 weeks
Ohtuvayre	$35,400.01		1553	3					12 weeks
Kisunla	$31,999.90		1736	3					76 weeks
Symvess	$29,500.00		54	2/3					30 days or 36 months
Cobenfy	$22,508.31		470	3					5 weeks

Brand Name	List Price	Total N Trials	Total N	Phase	Placebo Control	Active Control	Randomized	Single Arm	Timepoint
Vafseo	$15,549.00		3923	3					24 to 36 weeks
Rapiblyk	$12,950.00		1192	1, 2, 3					5 min, 10 min, or 24 to 72 hours
Sofdra	$8,784.51		701	3					6 weeks
Tryvio	$9,429.17		730	3					Up to 4 weeks
Orlynvah	$2,975.00		3861	3					12 days
Zelsuvmi	$1,950.00		1598	3					12 weeks
Letybo	$660.00		1271	3					4 weeks
Exblifep	‡		1041	3					7 days after end of treatment
Unloxcyt	‡		109	1					4 weeks or 6 months

N: number

*Dose ranging study

†Crossover study

‡List price not available

A2. Health System Impact Analysis

A2.1. Drugs in Scope

This section was based only on drugs in scope for the report that were previously reviewed by ICER among drugs that were approved in the US between 2022 and 2024. Of a total of 25 ICER-reviewed drugs, the following two drugs were excluded from the analysis: (1) Amvuttra: excluded because indications for ICER's review and the first FDA approval were different, and (2) Paxlovid: excluded because the drug was used before its full FDA approval under an EUA at prices negotiated by the US government. The list of included drugs can be found in Table 3.4.

A2.2. Framework

For drugs within our scope, we estimated excess drug spending during the first year post-approval and quantified the associated health opportunity costs using the analytical framework described below.

Excess Drug Spending

For ICER-reviewed drugs with net prices exceeding the ICER HBPB, we estimated excess drug spending attributable to pricing above the HBPB threshold. Total US net sales served as a proxy for total drug spending at actual net prices, consistent with the approach used in previous studies.[51] We used US net sales from the first full year following the first complete quarter after FDA approval to approximate first-year post-approval drug spending.

Counterfactual first-year drug spending—representing a scenario where launch net prices aligned with ICER's HBPB—was estimated by applying the ratio of the ICER HBPB to the actual net price, following the methodology of Yeung et al.[51] Both lower and upper bounds of the ICER HBPB were extracted from ICER final evidence reports for each drug.

For drugs priced above the ICER HBPB upper bound, excess first-year drug spending was calculated as the difference between actual first-year spending and counterfactual spending at the HBPB level. We reported total excess drug spending for all drugs exceeding the HBPB upper bound, with results stratified by meaningful subcategories selected for exploratory purposes (e.g., calendar year, gene and cell therapies vs. non-gene and cell therapies). Excess spending estimates are presented as ranges corresponding to the HBPB lower and upper bounds.

$$\boldsymbol{Excess\ Drug\ Spending} = \boldsymbol{Net\ Sales} * \left(\mathbf{1} - \frac{\boldsymbol{HBPB}}{\boldsymbol{Actual\ Net\ Price}}\right)$$

Opportunity Costs

Using the estimated excess first-year drug spending, we calculated the health benefits foregone due to drugs priced above the ICER HBPB (i.e., health opportunity costs). Health opportunity costs were estimated through three approaches: (a) equal value life years (evLYs) lost, (b) health insurance coverage loss and associated mortality, and (c) additional number of individuals with potential access to high-valued drugs.

Equal Value Life Years Lost

The evLYs lost due to excess first-year drug spending were calculated by dividing excess spending by the US health opportunity cost per evLY, following the approach used in Naci et al.[52] The evLY is a patient-centered measure of health gains commonly used in cost-effectiveness analysis that values the years of life added by a given intervention equally, no matter the person's health status.

$$\boldsymbol{evLY\ Foregone = \frac{Excess\ Drug\ Spending}{Opportunity\ cost\ per\ evLY}}$$

Health Insurance Coverage Loss and Associated Mortality

The number of individuals losing health insurance coverage due to excess drug spending and the resulting deaths were estimated using the methodology from Vanness et al.[25] The calculation proceeded in several steps:

1. Proportional premium increase: Annual premium increase per person was calculated by dividing total excess first-year drug spending by the number of insured US individuals. This amount was divided by the average annual premium to determine the proportional premium increase.

$$\boldsymbol{Absolute\ Premium\ Increase = \frac{Excess\ Drug\ Spending}{Total\ Number\ of\ Enrollees}}$$

$$\boldsymbol{Proportional\ Premium\ Increase = \frac{Absolute\ Premium\ Increase}{Average\ Annual\ Premium}}$$

   This calculation assumes that the total excess first-year drug spending is fully passed on as a premium increase. Accordingly, the outcomes from the analysis (i.e., the number of individuals who would lose insurance coverage and the associated mortality) reflect the scenario in which the excess spending is translated entirely into higher premiums.

   The calculation was stratified by age group (19–34, 35–54, 55–64, and 65 or older) and insurance type (employer-sponsored vs. individual) to account for heterogeneity in model

inputs (e.g., average annual premium). Final outcomes will be weighted according to the distribution of these subgroups.

2. Total insurance losses: The proportional premium increase was multiplied by the elasticity of insurance loss with respect to premium changes to estimate the probability of an individual losing coverage. This probability was then multiplied by the total number of insured individuals in the US to estimate the expected number of people who would become uninsured due to the premium increase.

$$\boldsymbol{Number\ Uninsured = Proportional\ Premium\ Increase * Premium\ Elasticy\ of\ Coverage * Total\ Number\ of\ \ Enrollees}$$

   Premium elasticity of coverage represents the percentage change in coverage for a 1% change in premiums. Because elasticity varies by age and insurance type, it was estimated separately for each age group (19–34, 35–54, 55–64, and 65 or older) and insurance type (employer-sponsored vs. individual).

3. Mortality impact: Expected deaths due to insurance loss were calculated by dividing the total number of individuals losing insurance by the number needed to lose insurance to cause one death in one year (NNL).

$$\boldsymbol{Number\ of\ Deaths = \frac{Number\ Uninsured}{NNL}}$$

Number of Individuals with Potential Access to High-valued Drugs

Finally, we estimated how many individuals could have potentially gained drug access if the total first-year excess spending were redirected to other treatments. This analysis examined potential access to high-value drugs that were priced within ICER's HBPB range among the ICER-reviewed drugs in our study scope. We calculated this by dividing the total first-year excess spending from all drugs priced above ICER's HBPB by the annual launch net price of each target drug, yielding the estimated number of additional patients who could receive treatment annually.

$$\boldsymbol{Number\ of\ Patients\ Potentially\ Treated = \frac{Excess\ Drug\ Spending}{Annual\ Net\ Price\ of\ a\ Drug}}$$

The primary results of these analyses were based on the base-case parameter values presented in Table A2.2. For evLY lost, health insurance coverage loss, and associated mortality, we conducted a probabilistic sensitivity analysis to account for uncertainty in the parameters used to calculate these outcomes.

A2.3. Data

Excess Drug Spending

US net sales data for each drug were obtained from multiple sources: SSR Health data or data directly submitted by manufacturers were used whenever available; if unavailable, manufacturer financial reports, Datamonitor, Biomedtracker, or IPD Analytics were used. US net sales from the first full year following the first full quarter after FDA approval were used to approximate drug spending over the first year post-approval. For two drugs, Lenmeldy and Voydeya, for which net sales data were unavailable from any source, we approximated first-year drug spending by multiplying the annual net price by the total number of patients receiving the treatment during the year which was obtained from the IPD Analytics.

The annual net price was obtained from multiple data sources (e.g., ASP, SSR Health, FSS). Details on the sources are provided in Section 2.

The upper and lower bounds of ICER HBPB were obtained from the ICER final evidence reports for each drug. The lower and upper bounds of ICER's HBPB correspond to prices at the $100,000 per quality-adjusted life year (QALY) threshold and the $150,000 per evLY threshold, respectively, from a US health care system perspective. If a modified societal perspective is used as a co-base case, the upper bound reflects the price at the $150,000 per evLY threshold from that modified societal perspective.

Opportunity Costs

The data used to estimate the opportunity costs and their sources are provided in Table A2.1.

Table A2.1. Model Inputs

Input	Base Case	Lower Bound*	Upper Bound*	Source
Opportunity Costs per evLY (USD)	$100,000	$50,000	$150,000	Vanness, 2021; ICER, 2023 [25,53]
Number of Enrollees in the US (N)				
The Number of Enrollees in the US: Age 19-34	58,146,000	46,516,800	69,775,200	US Census Bureau, 2023 [54]
The Number of Enrollees in the US: Age 35-54	74,511,000	59,608,800	89,413,200	US Census Bureau, 2023 [54]
The Number of Enrollees in the US: Age 55-64	38,214,000	30,571,200	45,856,800	US Census Bureau, 2023 [54]
The Number of Enrollees in the US: Age 65+	57,243,000	45,794,400	68,691,600	US Census Bureau, 2023 [54]

Input	Base Case	Lower Bound*	Upper Bound*	Source
The Number of Enrollees in the US: All Ages	228,114,000	182,491,200	273,736,800	US Census Bureau, 2023 [54]
Average Annual Premium (USD)				
ACA Marketplace: Age 19-34	$4,982	$3,819	$5,083	KFF, 2025; CMS, 2018 [55,56]
ACA Marketplace: Age 35-54	$7,113	$5,453	$7,256	KFF, 2025; CMS, 2018 [55,56]
ACA Marketplace: Age 55-64	$12,185	$9,341	$12,430	KFF, 2025; CMS, 2018 [55,56]
ESI: Age 19-34§	$8,435	$7,753	$8,906	KFF, 2023 [57]
ESI: Age 35-54§	$8,435	$7,753	$8,906	KFF, 2023 [57]
ESI: Age 55-64§	$8,435	$7,753	$8,906	KFF, 2023 [57]
Average premium: Age 65+†	$2,552	$2,041	$3,062	Medicare, 2025; Freed, 2023 [58,59]
Proportion with ESI among insured individuals, Age 19-64‡	68%	54%	82%	Claxton, 2024; US Census Bureau, 2023 [54,60]
Premium Elasticity of Coverage (%/%)				
Premium Elasticity of Coverage: ACA, Age 19-34	-1.50	-2.38	-0.62	Vanness, 2021 [25]
Premium Elasticity of Coverage: ACA, Age 35-54	-1.05	-1.78	-0.43	Vanness, 2021 [25]
Premium Elasticity of Coverage: ACA, Age 55-64	-0.70	-1.23	-0.28	Vanness, 2021 [25]
Premium Elasticity of Coverage: Age 65+	0.00	0.00	0.00	Assumption
Premium Elasticity of Coverage, ESI, Small Firms	-0.81	-0.94	-0.68	Abraham 2014 [61]
Premium Elasticity of Coverage, ESI, Medium Firms	-0.26	-0.36	-0.16	Abraham 2014 [61]
Premium Elasticity of Coverage, ESI, Large Firms	-0.12	-0.22	-0.03	Abraham 2014 [61]
% Small Firms Among all Firms in the US	61%	N/A	N/A	Abraham 2014 [61]
% Medium Firms Among all Firms in the US	15%	N/A	N/A	Abraham 2014 [61]

Input	Base Case	Lower Bound*	Upper Bound*	Source
% Large Firms Among all Firms in the US	24%	N/A	N/A	Abraham 2014 [61]
Weighted Elasticity, ESI	-0.56	N/A	N/A	Calculation
The Number of Individuals Needed to Lose Insurance to Result in One Death in One Year (N)	277.5	155.9	435.1	Vanness, 2021 [25]

ACA: Affordable Care Act, ESI: employer-sponsored insurance, N: number, N/A: Not applicable, US: United States, USD: United States Dollar

*Upper and lower bounds will be used for a probabilistic sensitivity analysis to account for uncertainty in the parameters

†Estimated using a weighted average of the annual premiums for Original Medicare ($2,661) and Medicare Advantage plans ($2,442).[58,59] The proportion of individuals over 65 enrolled in Medicare Advantage was estimated using two sources reporting total Medicare enrollment (~66 million) and total Medicare Advantage enrollment (~33 million).[62,63]

‡Based on the KFF report, 60.4% of all non-elderly adults have employer-sponsored insurance (ESI).[60] To estimate the proportion of insured individuals who have ESI, we adjusted this figure to account for the fraction of non-elderly adults who are uninsured in the US.[54]

§The annual premium for individuals with ESI represents the total premium, including both the employee's and employer's contributions.

For the health opportunity cost per evLY in the US, base-case analysis assumed $100,000 per evLY, with sensitivity analysis ranging from $50,000 to $150,000 per evLY. This base-case estimate is consistent with ICER's cost-effectiveness threshold range ($50,000-$150,000 per evLY or QALY) and the empirical US threshold estimated by Vanness et al. based on health opportunity costs ($104,000; 95% CI: $51,000 to $209,000).[25,53]

The estimation of the health insurance coverage loss and associated mortality required several data elements including: the number of insured individuals in the US, the average annual premium of the insured individuals in the US, elasticity of insurance loss in response to proportional premium increase, and the number of individuals needed to lose insurance to result in one death.

The number of insured individuals in the US was obtained from the 2023 US Census data, broken down by age group.[54]

For those younger than 65 years old, annual insurance premiums were estimated separately by insurance type (ACA Marketplace vs. employer-sponsored insurance) and by age group. For the ACA Marketplace, premiums were calculated using the average benchmark premium for a 40-year-old as the baseline, as reported by KFF.[55] We then estimated premiums for other age groups by applying the federal age-based premium ratios published by CMS to this 40-year-old reference premium.[56] For ESI, the average annual premium was obtained from the 2023 employer health benefits survey by KFF. [57]

We used the average annual premium across all single coverage plans as an approximation of the annual premium cost per individual covered by ESI across all age groups. The annual premium for individuals with ESI represents the total premium, including both the employee's and employer's contributions. We used the total premium because the elasticity estimates applied to it reflect the probability that a company will offer insurance in response to changes in the total premium (explained further below). Based on the KFF report, 60.4% of all non-elderly adults have ESI.[60] To estimate the proportion of insured individuals who have ESI, we adjusted this figure to account for the fraction of non-elderly adults who are uninsured in the US.[54]

For those equal to or older than 65 years old, the annual premium was estimated using a weighted average of the annual premiums for Original Medicare ($2,661) and Medicare Advantage plans ($2,442).[58,59] The proportion of individuals over 65 enrolled in Medicare Advantage was estimated using two sources reporting total Medicare enrollment (~66 million) and total Medicare Advantage enrollment (~33 million).[62,63]

The premium elasticity of coverage was also estimated separately by insurance type (ACA Marketplace vs. employer-sponsored insurance) and by age group. For non-elderly individuals who purchase insurance through the ACA Marketplace, elasticity estimates for each age group were obtained from Vanness et al.[25] For non-elderly individuals with ESI, elasticity estimates were obtained from Abraham et al., who measured employers' price-sensitivity in offering health insurance using the Medical Expenditure Panel Survey Insurance Component.[61] Since the study reported elasticity by firm size, we calculated a weighted average based on the firm size distribution reported in the same study. For individuals aged 65 or older, data were limited, but economic experts expected the probability of losing insurance due to a premium increase to be very small. Therefore, the elasticity was conservatively assumed to be zero.

A2.4. Additional Results

Probabilistic Sensitivity Analysis

Table A2.2. Probabilistic Sensitivity Analysis Results: Opportunity Costs of First-year Excess Drug Spending

	Lost evLY		Number of People Losing Insurance		Number of Deaths Due to the Insurance Loss	
	PSA Mean (95% CI)		PSA Mean (95% CI)		PSA Mean (95% CI)	
	Lower HBPB	Upper HBPB	Lower HBPB	Upper HBPB	Lower HBPB	Upper HBPB
All Drugs (N=17)	16,080 (10,148, 30,309)	13,609 (8,589, 25,652)	117,451 (79,911, 167,732)	99,402 (67,631, 141,956)	467 (237, 990)	395 (201, 838)
By Calendar Year						
2022 (N=5)	3,418 (2,157, 6,443)	2,585 (1,631, 4,873)	24,966 (16,986, 35,654)	18,882 (12,847, 26,965)	99 (50, 211)	75 (38, 159)
2023 (N=6)	1,672 (1,055, 3,152)	1,303 (822, 2,456)	12,213 (8,309, 17,441)	9,516 (6,475, 13,590)	49 (25, 103)	38 (19, 80)
2024 (N=6)	10,990 (6,936, 20,715)	9,721 (6,135, 18,323)	80,272 (54,616, 114,637)	71,004 (48,310, 101,400)	319 (162, 677)	282 (143, 599)

CI: credible interval, evLY: equal value life year, HBPB: health benefit price benchmark, N: number PSA: probabilistic sensitivity analysis

A2.5. Manufacturer Price Justification

To provide context on how the annual net price was determined, we included pricing justifications. For each drug reviewed by ICER, we located information on the pricing justification by the manufacturer. We obtained this information from 1) online sources (e.g., press releases from the manufacturer, news articles on the drug), or 2) requesting this information from the manufacturer. We present only data submitted by the manufacturer in the main report.

In this Appendix, we present data from both sources. If we received data from the manufacturer, we gave priority to the manufacturer's submitted data. We then categorized the justification into 10 categories, with illustrative examples provided below. Table A2.3 summarizes the pricing justification for each drug priced outside of ICER's HBPB (N=16). For five drugs (Cibinqo, Carvykti, Fabhalta, Voydeya, Rytelo), no pricing justification was provided or identified.

Table A2.3. Categories for Pricing Justification (Online Sources and Manufacturer Input): Individual Drug Data

Drug	Novelty	Clinical and Safety Profile	Internal Economic Model	Aligned with Clinical and Economic Value	Cost-Saving	Promote Patient Access	Priced Below/In Alignment with Competing Products	Stakeholder Input	Funding Future Research	No Comment
Veozah	★	★		★						
Leqembi			★	★		★				
Ohtuvayre	★			★						
Camzyos	★	★								
Briumvi							★			
Relyvrio*								★	★	
Attruby							★			
Winrevair	★	★		★		★			★	
Casgevy							★			
Roctavian		★			★					
Lyfgenia		★		★						
Lenmeldy				★						
Voydeya										⊘
Rytelo										⊘
Carvykti										⊘
Fabhalta										⊘

Examples of the price justification for each category are reported below. Examples with an asterisk are referencing pricing justification shared by the manufacturer versus those found in the public domain.

- ***Novelty*** e.g., "WINREVAIR is a significant innovation and the first FDA-approved activin signaling inhibitor therapy to treat PAH. In pricing WINREVAIR, we took many factors into account, including the value of this innovative therapy to patients and the healthcare system..."*
- ***Clinical and Safety Profile*** e.g., "Bluebird has set the wholesale acquisition cost of LYFGENIA in the U.S. at $3.1M in recognition of the value the therapy may deliver through robust and sustained clinical benefits"[64]
- ***Conducted Internal Economic Model*** e.g., "We assess the simulated impact of our medicines (Leqembi) on reducing demand for health services and global burden of disease as potential "economic value" while enhancing further innovations in [Alzheimer's disease]"[26]
- ***Aligned with Clinical and Economic Value*** e.g., "Rezdiffra demonstrated statistically significant and clinically meaningful improvements in both coprimary histologic endpoints recommended by the FDA and EMA [European Medicines Agency]: resolution of MASH without worsening of fibrosis, and at least 1-stage improvement in fibrosis without worsening of MASH…. Economically, its value is underscored by microsimulation modeling that projects a 50% reduction in the incidence of decompensated cirrhosis, hepatocellular carcinoma, and liver transplant, yielding substantial cost offsets"*
- ***Cost-saving*** e.g., For Roctavian, "BioMarin argues that its therapy would likely save the healthcare system money over time."[65]
- ***Promote Patient Access*** e.g., "Eisai decided to price LEQEMBI IV below quantified societal value at the wholesale acquisition cost (WAC) of $26,500 per year (estimated annual price based on 10mg/kg IV biweekly for average U.S. patient weight of 75kg based on Study 201 and Clarity AD) aiming to promote broader patient access, reduce overall financial burden, and support health system sustainability."*
- ***Priced Below/In Alignment with Competing Products*** e.g., "Vertex announced that the list price of Casgevy would be set at $2.2 million, placing it within the same roughly $2 million range as other recently approved gene therapies such as Novartis' Zolgensma® and Bluebird's Zynteglo®."[66]
- ***Stakeholder Input*** e.g., For Relyvrio, "(Co-chief executive officers) Klee and Cohen said they arrived at the price after discussions with patients, insurers and other stakeholders while also looking at funding future research"[67]

- ***Funding Future Research*** e.g., "In pricing WINREVAIR, we took many factors into account, including the value of this innovative therapy to patients and the healthcare system and our plans for continued investment in clinical research while balancing access and affordability needs for patients."

B. Access: Detailed Methodology

We evaluated patient access to drugs approved in 2024. To do so, we obtained data from two data source (Tufts Medical Center Specialty Drug Evidence and Coverage [SPEC] Database and IQVIA) and collaborated with the National Health Council (NHC) to collect qualitative feedback from patient groups about patient experience accessing newly launched drugs.

We obtained data from the SPEC Database. This data includes information on specialty drug coverage decisions issued by up to 18 large US commercial health plans. At the December 2024 data cutoff, we had access to 18 drugs within scope of our report in the SPEC Database, with a total of 324 potential policies. There were 163 policies available (50%). At the April 2025 data cutoff, we had access to 24 drugs within scope of our report in the SPEC Database, with a total of 451 potential policies. There were 267 policies available (59%). We also obtained coverage data on Zepbound (approved in 2023) which has 13 policies available (out of 18 commercial payer policies) at the data cutoff date of April 2025.

We also obtained data from IQVIA, a leading health care data and analytics provider. They provided measures on consumer access through their Longitudinal Access and Adjudication Data (LAAD), which included information on prior authorization analytics and patient out-of-pocket costs for both commercially-insured and cash paying patients. For the IQVIA data, we focused on first quarter 2025 metrics which were available for 28 of the 55 drugs in scope for the 2024 approval year. We excluded 11 of 28 drugs that had <100 total commercial written prescriptions for the quarter to ensure numeric trends were not caused by randomness, leaving 17 drugs in the analysis. Given our interest in obesity medicines, data for Zepbound was also included in the package, despite it being approved in 2023.

Patient and patient advocate attendees for the patient group discussions were recruited from previous ICER review engagements and supplemented with National Health Council (NHC) membership (Table B1.1). There were two facilitated group discussions and one individual patient discussion, all conducted virtually over Zoom:

A. Group 1 (n=4): Common conditions, with the exception of PAH which was grouped into Group 1 to complement the COPD findings
B. Group 2 (n= 4): Rare conditions
C. Individual Patient Discussion (n=1): PNH

NHC led recruitment efforts which began in June 2025, and all three sessions were conducted in July 2025. A moderator from NHC facilitated all discussions.

Table B1.1. Conditions Discussed in Facilitated Group Discussions

Condition	Drug Brand
Anemia in Myelodysplastic Syndrome (MDS)	Rytelo
Cardiomyopathy of Transthyretin-Mediated Amyloidosis (ATTR-CM)	Attruby
Chronic Obstructive Pulmonary Disease (COPD)	Ohtuvayre
Metachromatic Leukodystrophy (MLD)	Lenmeldy
Metabolic Dysfunction-Associated Steatohepatitis (MASH)	Rezdiffra
Paroxysmal Nocturnal Hemoglobinuria (PNH)	Voydeya
Pulmonary Arterial Hypertension (PAH)	Winrevair
Schizophrenia	Cobenfy

B1. Initial Coverage Policy Availability

To evaluate the availability of coverage policies in the first 6-12 months after approval, we used data from the Tufts Medical Center Specialty Drug Evidence and Coverage (SPEC) Database. From this source, we obtained data for the proportion of health plans with coverage for each selected drug, proportion of health plans that explicitly did not cover the drug, and proportion of health plans that did not have a policy available and/or policy was not located by the SPEC team. We obtained data from two timepoints: December 2024 data cutoff and April 2025 data cutoff.

B2. Prior Authorization Burden

Data obtained from IQVIA on included total written and dispensed commercial prescriptions from their Longitudinal Access & Adjudication Data (LAAD) which is sourced from open-source pharmacy claims. Based upon total new-to-brand prescriptions, percentages of claims that were filled, rejected, and abandoned were available for all attempts at coverage. New-to-brand claims represent a patient's first prescription of a drug. Rejected claims were further broken down by reasons, including non-coverage, need for prior authorization, step therapy, or administrative errors. We obtained data on percentage filled, percentage rejected due to non-coverage, prior authorization/step therapy, or other reasons (e.g., fill limit, etc.), and percentage reversed (e.g., patient did not collect the prescription). These percentages were available for single, multiple, and all attempts.

Table B2.1. Attributes of Novel FDA Approvals in 2024 that Have Q1 2025 Data Available from IQVIA's Consumer Access Report

Drug Name	Approval Date	Therapeutic Area	First-in-Class	Orphan	Priced to Value*
Zepbound	11/8/2023	Endocrine/Metabolic	No	No	-
Rezdiffra	3/14/2024	Digestive	Yes	No	Yes
Iqirvo	6/10/2024	Digestive	Yes	Yes	-
Sofdra	6/18/2024	Dermatology	No	No	-
Ohtuvayre	6/26/2024	Respiratory	No	No	No
Voranigo	8/6/2024	Oncology	No	Yes	-
Yorvipath	8/9/2024	Endocrine/Metabolic	No	Yes	-
Nemluvio	8/12/2024	Dermatology	Yes	No	-
Livdelzi	8/14/2024	Digestive	No	Yes	-
Lazcluze	8/19/2024	Oncology	No	No	-
Ebglyss	9/13/2024	Dermatology	No	No	-
Miplyffa	9/20/2024	Endocrine/Metabolic	Yes	Yes	-
Aqneursa	9/24/2024	Neurology	Yes	Yes	-
Cobenfy	9/26/2024	Mental/Behavioral health	Yes	No	Yes
Itovebi	10/10/2024	Oncology	No	No	-
Revuforj	11/15/2024	Oncology	Yes	Yes	-
Attruby	11/22/2024	Cardiovascular	No	Yes	No
Alyftrek	12/20/2024	Respiratory	No	Yes	-

*As determined by a drug's net price in relation to the ICER Health-Benefit Price Benchmark.

Table B2.2. IQVIA Data on Prior Authorization Burden (Q1 2025 Data). Percentages Based on New-to-Brand Dispensed Claims. Commercial Pharmacy Claims Only.

Drug Name	Total Commercial Written Prescriptions	Total Commercial New-to-Brand Dispensed	Covered			Rejected		
			Filled	Abandoned	Overall	Not Covered	PA/ST/Other	Overall
Rezdiffra	6,679	1,158	6%	19%	25%	30%	46%	76%
Iqirvo	730	164	34%	6%	40%	23%	37%	60%
Sofdra	1,682	676	27%	3%	30%	60%	10%	70%
Ohtuvayre	715	124	5%	20%	25%	29%	47%	76%
Voranigo	1,285	189	39%	29%	68%	11%	21%	32%
Yorvipath	152	37	36%	17%	53%	30%	17%	47%
Nemluvio	4,588	1,173	23%	3%	26%	54%	21%	75%
Livdelzi	1,617	327	51%	8%	59%	25%	16%	41%
Lazcluze	212	36	46%	15%	61%	20%	20%	40%
Ebglyss	3,093	706	19%	8%	27%	55%	19%	74%
Miplyffa	102	5	0%	50%	50%	50%	0%	50%
Aqneursa	207	18	13%	7%	20%	73%	7%	80%
Cobenfy	2,526	694	36%	8%	44%	41%	14%	55%
Itovebi	212	57	41%	11%	52%	27%	20%	47%
Revuforj	120	26	36%	16%	52%	28%	20%	48%
Attruby	218	57	27%	16%	43%	36%	21%	57%
Alyftrek	486	211	35%	4%	39%	40%	21%	61%

B3. Patient Cost Sharing

IQVIA data included out-of-pocket costs for both commercially-insured and cash paying prescriptions. These measures were displayed as percentages of claims that fall into categories of costs as opposed to specific dollar amounts. All out-of-pocket cost information is based on the total number of written prescriptions.

Table B3.1. IQVIA Consumer Access Analytics for Cash-Pay Prescriptions in Q1 2025, Including Patient Out-of-Pocket Costs. Third-Party Commercial, Government Payer, or Coupon/Discount Cards are Excluded.

Drug Name	Monthly List Price	Total Cash Written Prescriptions	Total Cash New-to-Brand Dispensed	Patient Monthly OOP Cost (Cash Pay)*				
				$0	$1-$50	$51-$250	$250-$1,500	$1,500+
Rezdiffra	$4,004.86	39	11	9%	0%	0%	0%	91%
Sofdra	$1,464.09	21	9	-	0%	0%	27%	73%
Ohtuvayre	$2,950.00	4	-	-	0%	0%	25%	75%
Voranigo	$40,434.90	1	1	0%	0%	0%	0%	100%
Nemluvio	$4,593.33	4	1	25%	0%	0%	0%	75%
Lazcluze	$18,450.75	2	1	0%	0%	0%	0%	100%
Ebglyss	$5,541.67	35	10	0%	7%	0%	12%	81%
Aqneursa	$58,443.45	3	2	0%	0%	0%	0%	100%
Cobenfy	$1,875.69	78	40	12%	1%	5%	24%	58%
Itovebi	$24,840.64	4	-	0%	0%	0%	0%	100%
Revuforj	$67,582.03	1	-	0%	0%	0%	0%	100%
Attruby	$20,378.21	5	-	0%	0%	0%	0%	100%
Alyftrek	$30,855.77	1	-	0%	0%	0%	0%	100%

*Percentages are based on total cash written prescriptions

B4. Patient Experience

Eight organization representatives and one individual patient were invited to participate and all nine accepted. All participants were given the opportunity to complete a "Pre-Survey" to rank access challenges and facilitators for their community and provide any additional context. Nine responses total from seven conditions were submitted and helped in the development of a discussion guide used for the facilitated group discussions. Topics introduced during the facilitated group discussions included barriers to access, impact of new treatments on patient access, and impact of patient assistance programs, accelerators, or copay card programs on access for their respective communities. The two group discussions each had four participants, with one additional participant interviewed one-on-one after the focus groups concluded. All patient/caregiver/patient advocate participants were offered compensation in the form of an honorarium of $200 each. This is in line with the NHC's patient engagement Fair-Market Value Calculator. The discussion guide was developed by ICER and the NHC; questions were focused on top barriers to care and access to medications that had been approved by the FDA in 2024.