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Methodological Frameworks for Evaluating Patient Access Barriers to High-Cost Therapies in Medicaid and Medicare Populations

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ABSTRACT

The rapid expansion of high-cost therapies within public insurance programs has intensified concerns regarding affordability, sustainability, and equitable patient access. This study develops a structured comparative methodological framework to evaluate patient access barriers to high-cost therapies across Medicaid and Medicare populations. Five dominant evaluative approaches are examined: cost-effectiveness and health technology assessment models, budget impact analyses, real-world evidence integration frameworks, payment innovation models, and behavioral disparity-oriented methodologies. The comparative analysis reveals that no single framework sufficiently captures all dimensions of access barriers. Cost-effectiveness models provide strong long-term value estimation but require recalibration for one-time curative therapies with high upfront costs. Budget impact models support short-term fiscal planning yet frequently overlook equity dynamics. Real-world evidence frameworks enhance policy adaptability, while spread payment and insurance redesign models mitigate financial volatility but introduce administrative complexity. Behavioral and disparity-focused frameworks uniquely detect structural inequities invisible to purely economic approaches. The study concludes that integrated hybrid methodological approaches, combining economic valuation, fiscal forecasting, real-world data integration, payment reform assessment, and equity-sensitive metrics, provide the most comprehensive structure for evaluating access barriers and informing policy reform within evolving Medicaid and Medicare systems.

Keywords: cost-effectiveness, health technology assessment, Medicaid, Medicare, gene therapy, real-world evidence, health equity

1.0 INTRODUCTION

The rapid growth of high-cost therapies including gene therapies, cell-based interventions, specialty oncology drugs, and digital therapeutics has transformed treatment possibilities across multiple disease areas. However, their high upfront prices and complex reimbursement pathways pose significant challenges for public insurance programs, particularly Medicaid and Medicare (Trish et al., 2021). As enrollment in these programs continues to expand, ensuring equitable and sustainable access to innovative therapies has become a pressing policy concern (Keisler-Starkey & Bunch, 2023).

Medicaid and Medicare beneficiaries often face distinct access barriers, including coverage variability across states, prior authorization requirements, cost-sharing burdens, reimbursement constraints, and administrative complexities (Allen et al., 2023; Auletta et al., 2023).

Evaluating patient access barriers in this context requires robust methodological frameworks that integrate health technology assessment (HTA), cost-effectiveness analysis, budget impact modeling, real-world evidence (RWE), and policy evaluation tools (Basu et al., 2024; Angelis et al., 2020). Recent studies have also highlighted the importance of behavioral and system-level perspectives in understanding access. Patient engagement frameworks demonstrate how administrative burden, complexity, and financial strain influence treatment uptake (Kimerling et al., 2020). Similarly, policy evaluations of Medicaid accountable care organizations and oncology care models reveal how system design shapes utilization and cost outcomes (Holm et al., 2024; Theroux et al., 2020).

Despite these advances, the literature remains fragmented. Existing studies often focus narrowly on reimbursement policy, economic evaluation, or clinical outcomes without integrating these domains into a unified methodological structure. Furthermore, variations in market access decisions across jurisdictions underscore the absence of standardized evaluation approaches (Tunis et al., 2021).

This review addresses this gap by synthesizing evidence from peer-reviewed and policy-oriented studies to develop a structured methodological framework for evaluating patient access barriers to high-cost therapies in Medicaid and Medicare populations. Specifically, this study:

1. Identifies core domains of access barriers
2. Compares existing methodological approaches used to assess these barriers.
3. Proposes an integrated evaluative framework that aligns economic modeling, real-world evidence, and policy analytics.

Using a structured comparative approach, this review seeks to equip policymakers, researchers, and payers with a coherent analytical lens to assess and reform access pathways for high-cost therapies within U.S. public insurance systems.

2.0 METHODOLOGY

2.1 Study Design

This study employs a structured comparative review methodology to evaluate and synthesize methodological frameworks used to assess patient access barriers to high-cost therapies within Medicaid and Medicare populations. The approach integrates elements of scoping review methodology (Arksey & O'Malley, 2005; Tricco et al., 2018) with structured comparative analysis, enabling systematic identification of framework strengths, limitations, and complementarities across multiple evaluative dimensions.

Unlike traditional systematic reviews focused on clinical efficacy or single-outcome measurement, this study adopts a framework-level unit of analysis. Each methodological approach is treated as an evaluative system and assessed for its capacity to capture access barriers within public insurance environments characterized by fiscal constraints, administrative complexity, and demographic diversity.

2.2 Search Strategy

A comprehensive literature search was conducted across multiple electronic databases, including PubMed, Scopus, Web of Science, EconLit, and Google Scholar, covering publications from January 2019 through December 2025. The search was supplemented by targeted review of gray literature from policy-oriented sources, including reports from the Centers for Medicare and Medicaid Services (CMS), the Milbank Quarterly, Health Affairs, and the Institute for Clinical and Economic Review (ICER).

Search terms were organized into three conceptual clusters combined using Boolean operators:

- **Population and setting:** "Medicaid," "Medicare," "public insurance," "public payer," "U.S. health coverage"
- **Intervention context:** "high-cost therapy," "gene therapy," "cell therapy," "specialty drug," "regenerative medicine," "digital therapeutics," "biologic," "CAR-T"
- **Methodological focus:** "cost-effectiveness analysis," "health technology assessment," "budget impact," "real-world evidence," "outcome-based payment," "spread payment," "insurance design," "access barriers," "health equity," "coverage policy," "predictive analytics"

Reference lists of included studies were also hand-searched to identify additional relevant sources.

2.3 Inclusion and Exclusion Criteria

Studies were included if they met the following criteria:

- Addressed methodological approaches to evaluating access, coverage, reimbursement, or utilization of high-cost therapies within Medicaid, Medicare, or comparable U.S. public insurance contexts
- Presented or applied evaluative frameworks, models, or analytical tools relevant to patient access assessment (including economic evaluation, policy analysis, real-world evidence application, payment reform modeling, or behavioral and equity-focused analysis)
- Were published in English between January 2019 and December 2025
- Appeared in peer-reviewed journals or recognized policy/research institutions

Studies were excluded if they:

- Focused exclusively on clinical efficacy without addressing access, coverage, or reimbursement dimensions
- Addressed private or employer-sponsored insurance without relevance to Medicaid or Medicare
- Were editorials, commentaries, or opinion pieces without empirical, analytical, or conceptual methodological content

2.4 Source Selection and Screening

An initial search yielded approximately 180 records. After removing duplicates, titles and abstracts of approximately 130 unique records were screened for relevance to the study's focus on methodological evaluation of access barriers. Full texts of potentially relevant studies were then reviewed against the inclusion criteria. A final set of 29 sources was retained for analysis. While a formal PRISMA flow diagram was not employed, the screening process followed a structured and iterative approach consistent with scoping review principles (Tricco et al., 2018).

2.5 Analytical Framework

To enable structured cross-framework comparison, included studies were categorized into five methodological classes based on their primary evaluative orientation:

1. **Cost-effectiveness and health technology assessment (HTA) models** — frameworks centered on economic efficiency, including incremental cost-effectiveness ratio (ICER) analysis and quality-adjusted life year (QALY) estimation
2. **Budget impact and expenditure models** — frameworks focused on short-term fiscal forecasting and aggregate spending projections within public payer budgets
3. **Real-world evidence (RWE) integration frameworks** — approaches incorporating claims data, post-market utilization trends, and observational outcome data into access and value assessment
4. **Payment innovation and insurance redesign models** — proposals for outcome-based spread payments, federal reinsurance mechanisms, and alternative financing structures

5. **Behavioral and disparity-focused analytical frameworks** — approaches emphasizing patient engagement dynamics, administrative burden, and equity-sensitive metrics such as racial, ethnic, and income-based disparities in prescribing and adherence

Each framework class was then assessed across six standardized evaluation dimensions derived from the reviewed literature:

Dimension	Definition
Long-Term Value Estimation	Capacity to project lifetime clinical and economic outcomes of high-cost therapies
Short-Term Fiscal Forecasting	Ability to model immediate budgetary impacts within annual Medicaid and Medicare cycles
Equity Sensitivity	Effectiveness in detecting disparities in access, prescribing, and adherence across demographic groups
Adaptability to Policy Reform	Responsiveness to shifts in reimbursement policy, coverage criteria, or regulatory environments
Administrative Feasibility	Practicability of implementation given existing data infrastructure and administrative capacity
Scalability Across States	Applicability across diverse state Medicaid programs with varying coverage rules and administrative structures

Performance ratings (High, Moderate, Low, Very High) were assigned based on a qualitative synthesis of evidence from the reviewed literature. A rating of "High" indicates that the reviewed evidence consistently supports strong framework performance on a given dimension across multiple studies or policy contexts. "Moderate" reflects mixed or context-dependent evidence, while "Low" indicates limited demonstrated capacity. "Very High" is reserved for cases where the reviewed literature identifies a framework as distinctly superior on a specific dimension relative to all other approaches.

2.6 Limitations of the Methodology

Several limitations should be acknowledged. First, the comparative ratings are derived from qualitative synthesis rather than quantitative meta-analysis, introducing a degree of interpretive judgment. Second, the search strategy, while comprehensive, may not have captured all relevant gray literature or unpublished policy analyses. Third, the five-category framework typology, while analytically useful, necessarily simplifies the diversity of hybrid and emerging approaches in literature. Finally, rapid evolution in CMS reimbursement policy and state Medicaid coverage practices means that findings reflect the policy landscape as of early 2025 and may require periodic reassessment.

3.0 LITERATURE REVIEW

3.1 Overview of High-Cost Therapies in Medicaid and Medicare

Innovative high-cost treatments such as gene therapies, regenerative medicines, specialty oncology agents, and emerging digital therapeutics have delivered transformative clinical gains while simultaneously intensifying financial strain on Medicaid and Medicare programs. Specialty drug spending continues to rise across both populations, driven largely by biologics and advanced therapies (Trish et al., 2021). Medicare beneficiaries, in particular, face increasing out-of-pocket obligations associated with high-cost medications, even when covered under Part B or Part D (Conti et al., 2025).

Within Medicaid, coverage practices vary significantly by state, creating inconsistencies in patient access to approved gene and cell therapies (Allen et al., 2023). Analyses of hematopoietic cell transplantation and CAR-T therapy reveal structural coverage limitations, prior authorization barriers, and reimbursement delays that directly affect beneficiary access (Auletta et al., 2023). Furthermore, policy discussions surrounding oncologic drug reimbursement and proposed cancer care reforms indicate that reimbursement design significantly shapes availability and provider participation (Huey et al., 2025; Theroux et al., 2020). Beyond financial design, demographic and insurance coverage trends influence access. Medicaid expansion and coverage distribution patterns remain central determinants of eligibility and treatment uptake (Keisler-Starkey & Bunch, 2023). However, disparities persist. Racial, ethnic, and income-based differences in medication prescribing patterns among Medicare beneficiaries highlight inequities embedded within existing access pathways (Friedman et al., 2021). Cost-related medication nonadherence further illustrates how financial burden translates into reduced therapeutic utilization (Kizza, Aduampong, & Kaiser, 2025)

3.2 Economic Evaluation and Health Technology Assessment Frameworks

A central methodological pillar in evaluating access barriers is health technology assessment (HTA). Traditional HTA frameworks, which rely heavily on incremental cost-effectiveness ratios, have been recalibrated to address the unique economic and evidentiary characteristics of gene and cell therapies (Angelis et al., 2020; Coyle et al., 2020). These therapies often involve high upfront costs, long-term uncertainty, and small patient populations conditions that challenge standard cost-effectiveness thresholds. Cost-effectiveness modeling has been applied to evaluate gene therapy relative to conventional care, as demonstrated in analyses of sickle cell disease (Basu et al., 2024). Such models integrate long-term clinical projections, quality-adjusted life years (QALYs), and payer perspectives to assess value within Medicare and Medicaid contexts. Similarly, budget impact analyses of biosimilars illustrate how alternative pricing structures may reduce financial strain on Medicare populations (Yang et al., 2021). However, variation in market access decisions across jurisdictions demonstrates inconsistency in value interpretation and reimbursement standards (Tunis et al., 2021). This variation suggests the need for standardized methodological approaches capable of integrating clinical evidence, economic modeling, and equity considerations within public insurance systems.

Recent contributions also emphasize the growing importance of real-world evidence (RWE) in value assessment and pricing negotiations. Frameworks for quantifying healthcare value using real-world data expand beyond traditional trial-based evidence, allowing dynamic evaluation of outcomes and utilization patterns (Pyenson et al., 2024). Tunis et al. (2025) further noted that the incorporation of RWE into Medicare drug price negotiation processes underscores the methodological shift toward data-driven reimbursement policy.

3.3 Innovative Payment and Insurance Design Models

Given the structural misalignment between high upfront costs and annual budget cycles, scholars have proposed alternative reimbursement mechanisms to mitigate access barriers. Outcome-based spread payments allow costs of one-time curative therapies to be distributed over time, contingent upon observed clinical performance (Michelsen et al., 2020). These models seek to reduce financial risk for state Medicaid programs while preserving access to transformative therapies.

Innovative insurance design models similarly propose risk-pooling mechanisms and federal reinsurance approaches to stabilize funding for gene and cell therapies (Conti et al., 2025). Such proposals aim to reconcile fiscal sustainability with equitable beneficiary access. At the regulatory level, broader analyses

of challenges in regenerative medicine market access highlight the interplay between manufacturing complexity, reimbursement uncertainty, and regulatory oversight (Qiu et al., 2022; Musyuni et al., 2025). Prescription digital therapeutics represent an additional frontier in coverage evaluation, where Medicaid experience has informed formulary management and value assessment methodologies (Kizza & Oware, 2025). These emerging therapeutic categories further complicate access evaluation, requiring adaptive methodological tools.

3.4 Policy Evaluation and System-Level Frameworks

Beyond economic modeling, methodological frameworks increasingly incorporate system-level policy evaluation tools. Predictive analytics has been applied to model medication outcomes and policy impacts within Medicaid populations, illustrating how data-driven simulation can forecast access consequences under alternative policy scenarios (Adetunji, 2025). Evaluations of Medicaid accountable care organizations (ACOs) demonstrate how care coordination and payment reform influence utilization, quality measures, and costs over time (Holm et al., 2024). Additionally, analyses of dental coverage discontinuities ("coverage cliffs") among low-income Medicare beneficiaries show how policy design directly shapes service utilization patterns (Whidden, 2022).

Behavioral frameworks further contextualize access barriers by conceptualizing patient engagement as either opportunity or burden, depending on system complexity and support structures (Kimerling et al., 2020). Such perspectives emphasize that administrative burden and informational barriers can operate alongside financial constraints to limit access. Moreover, assessments of CMS quality measures reveal potential misalignment between quality metrics and cost-effectiveness evidence, indicating methodological gaps between performance measurement and value assessment (Dover & Kim, 2021).

3.5 Gaps in Existing Methodological Approaches

Despite substantial research, key gaps remain. First, economic evaluations frequently operate independently of behavioral and administrative analyses, limiting comprehensive understanding of access barriers. Second, while cost-effectiveness models provide insights into value, they often underrepresent real-world implementation challenges, including coverage variability and regulatory complexity. Third, equity considerations such as racial disparities and income-based nonadherence are inconsistently integrated into evaluative frameworks (Friedman et al., 2021; Kizza, Aduampong, & Kaiser, 2025).

The literature underscores the need for an integrated methodological structure that synthesizes HTA, real-world evidence, predictive analytics, payment innovation, and behavioral frameworks to evaluate patient access barriers comprehensively within Medicaid and Medicare populations.

4.0 COMPARATIVE ANALYSIS AND RESULTS

4.1 Performance of Methodological Frameworks Under Baseline Policy Conditions

A structured comparative evaluation of methodological frameworks used to assess patient access barriers in Medicaid and Medicare populations reveals distinct differences across several dimensions, including analytical depth, fiscal sensitivity, scalability, and equity responsiveness. This analysis identifies five dominant framework classes: (1) cost-effectiveness and health technology assessment (HTA) models, which prioritize economic efficiency; (2) budget impact and expenditure modeling, focused on financial forecasting; (3) real-world evidence (RWE) integration frameworks, emphasizing practical data application; (4) payment innovation and insurance redesign models, aimed at structural reforms; and (5) policy-behavioral and disparity-focused analytical frameworks, which address equity and behavioral dynamics.

Under baseline policy conditions, defined as current CMS reimbursement structures, standard cost-sharing provisions, and existing state Medicaid coverage variation, cost-effectiveness models demonstrate strong performance in estimating long-term therapeutic value. For example, gene therapy evaluation models for sickle cell disease illustrate how lifetime QALY projections may justify high upfront costs when compared to chronic care pathways (Basu et al., 2024). However, recalibration analyses highlight that traditional ICER thresholds may inadequately reflect curative intent therapies (Angelis et al., 2020; Coyle et al., 2020).

Budget impact analyses, particularly those examining specialty drug spending trends, provide immediate fiscal forecasting capability for Medicare and Medicaid programs (Trish et al., 2021; Conti et al., 2025). Yet these approaches prioritize short-term expenditure containment and may underrepresent long-term outcome gains. Real-world evidence frameworks enhance evaluative realism by incorporating claims data, longitudinal utilization trends, and outcome heterogeneity (Pyenson et al., 2024; Tunis et al., 2025). These models demonstrate improved adaptability in Medicare drug price negotiation environments but require robust data infrastructure.

Payment innovation models such as outcome-based spread payments and federal reinsurance concepts demonstrate superior fiscal smoothing performance under high upfront cost scenarios (Michelsen et al., 2020; Conti et al., 2025). However, implementation complexity and administrative burden may limit scalability within fragmented state Medicaid systems. Behavioral and disparity-oriented frameworks provide essential insight into cost-related nonadherence and racial/ethnic prescribing inequities (Friedman et al., 2021; Kizza, Aduampong, & Kaiser, 2025). While these models may lack fiscal forecasting depth, they outperform purely economic models in identifying equity-sensitive barriers.

4.2 Performance Across Key Evaluation Dimensions

Table 1: Cross-Framework Comparative Assessment of Methodological Approaches

The following table presents a qualitative comparative assessment of five methodological framework classes across six standardized evaluation dimensions. Performance ratings were derived through structured synthesis of the reviewed literature, applying the rating scale defined in Section 2.5. Each rating reflects the weight and consistency of evidence across the included studies, as summarized in the justification notes below the table.

Criteria	CEA & HTA Models	Budget Impact Models	RWE Frameworks	Payment Innovation Models	Behavioral & Disparity Models
Long-Term Value Estimation	High	Low–Moderate	Moderate	Moderate	Low
Short-Term Fiscal Forecasting	Moderate	High	Moderate	High	Low
Equity Sensitivity	Moderate	Low	High	Moderate	Very High
Adaptability to Policy Reform	Moderate	Moderate	High	High	Moderate
Administrative Feasibility	High	High	Moderate	Low–Moderate	High

Criteria	CEA & HTA Models	Budget Impact Models	RWE Frameworks	Payment Innovation Models	Behavioral & Disparity Models
Scalability Across States	Moderate	High	Moderate	Low	High

Sources: Angelis et al., 2020; Basu et al., 2024; Conti et al., 2025; Coyle et al., 2020; Friedman et al., 2021; Kimerling et al., 2020; Kizza et al., 2025; Michelsen et al., 2020; Pyenson et al., 2024; Trish et al., 2021; Tunis et al., 2025

Rating Justifications by Framework and Dimension

1. Cost-Effectiveness and HTA Models

- **Long-Term Value Estimation: High.** CEA models are specifically designed for projecting lifetime outcomes. Gene therapy evaluations for sickle cell disease demonstrate the capacity to model quality-adjusted life years (QALYs) and lifetime cost offsets relative to chronic care pathways (Basu et al., 2024). Recalibration studies further confirm that, despite limitations with curative therapies, CEA remains the strongest available tool for long-term value estimation (Angelis et al., 2020; Coyle et al., 2020).
- **Short-Term Fiscal Forecasting: Moderate.** CEA models can generate cost projections but are not designed primarily for annual budget cycle planning. Their focus on lifetime horizons and discounting conventions limits direct applicability to short-term Medicaid and Medicare fiscal management.
- **Equity Sensitivity: Moderate.** Standard CEA frameworks can incorporate subgroup analyses and distributional weighting but do so inconsistently in practice. Equity dimensions are typically secondary to efficiency metrics in traditional HTA applications.
- **Adaptability to Policy Reform: Moderate.** CEA models can be updated with revised inputs but are structurally less responsive to rapid policy shifts than data-driven or RWE approaches. Threshold recalibration for curative therapies indicates some adaptability (Angelis et al., 2020).
- **Administrative Feasibility: High.** CEA and HTA methods are well-established, widely understood by payers, and supported by existing infrastructure at agencies such as ICER and CMS.
- **Scalability Across States: Moderate.** While the methodology is transferable, input parameters (e.g., Medicaid reimbursement rates, population demographics) vary by state, requiring localized adaptation.

2. Budget Impact Models

- **Long-Term Value Estimation: Low–Moderate.** Budget impact analyses focus on aggregate short-term spending and are not designed to capture lifetime clinical benefits or QALY gains. They may acknowledge long-term value indirectly but lack the modeling depth of CEA (Trish et al., 2021).
- **Short-Term Fiscal Forecasting: High.** This is the primary purpose of budget impact models. Studies of specialty drug spending trends demonstrate strong performance in projecting near-term expenditure growth for Medicare and Medicaid (Trish et al., 2021; Conti et al., 2025).
- **Equity Sensitivity: Low.** Budget impact analyses typically operate at the aggregate population level and do not stratify by race, ethnicity, or income. They may detect overall spending shifts but fail to identify disparities in who benefits from or is excluded by coverage decisions (Kizza, Aduampong, & Kaiser, 2025).

- **Adaptability to Policy Reform: Moderate.** Budget models can be recalculated under revised policy assumptions, but their structural simplicity limits nuanced scenario modeling.
- **Administrative Feasibility: High.** Budget impact analyses are standard components of formulary and coverage decision-making and require relatively straightforward data inputs.
- **Scalability Across States: High.** The methodology is easily applied across jurisdictions using state-specific expenditure data, making it one of the most scalable approaches.

3. Real-World Evidence (RWE) Frameworks

- **Long-Term Value Estimation: Moderate.** RWE can track longitudinal outcomes and treatment durability but is subject to observational biases and data completeness limitations. It supplements rather than replaces formal CEA modeling for long-term value estimation (Pyenson et al., 2024).
- **Short-Term Fiscal Forecasting: Moderate.** Claims-based RWE can inform utilization trends and spending patterns but is typically retrospective, limiting its use for prospective fiscal forecasting.
- **Equity Sensitivity: High.** RWE datasets can be stratified by demographic variables, enabling detection of disparities in utilization, prescribing, and outcomes across beneficiary subgroups (Pyenson et al., 2024; Tunis et al., 2025).
- **Adaptability to Policy Reform: High.** RWE frameworks are inherently responsive to policy changes because they draw on continuously updated administrative and claims data. Their incorporation into Medicare drug price negotiations reflects this adaptive capacity (Tunis et al., 2025).
- **Administrative Feasibility: Moderate.** RWE integration requires robust data infrastructure, linkage capabilities, and analytical expertise that may not be uniformly available across state Medicaid programs.
- **Scalability Across States: Moderate.** Data availability, quality, and interoperability vary significantly across states, constraining uniform application.

4. Payment Innovation and Insurance Redesign Models

- **Long-Term Value Estimation: Moderate.** Outcome-based spread payments implicitly incorporate long-term outcome expectations by linking payment to clinical performance over time (Michelsen et al., 2020). However, they are financing mechanisms rather than value estimation tools.
- **Short-Term Fiscal Forecasting: High.** Spread payment and reinsurance models are explicitly designed to smooth high upfront costs across budget cycles, directly addressing short-term fiscal volatility (Michelsen et al., 2020; Conti et al., 2025).
- **Equity Sensitivity: Moderate.** These models can expand access broadly by reducing financial barriers, but do not inherently incorporate equity metrics or target disparities in prescribing or adherence.
- **Adaptability to Policy Reform: High.** Payment innovation models are designed as policy reforms and are structurally flexible in accommodating new therapeutic categories and reimbursement structures.
- **Administrative Feasibility: Low–Moderate.** Implementation requires outcome tracking systems, intergovernmental coordination, and contractual infrastructure that represent significant administrative demands, particularly in fragmented state Medicaid systems (Michelsen et al., 2020).
- **Scalability Across States: Low.** Variability in state administrative capacity, political conditions, and Medicaid program structures limits uniform adoption. No standardized implementation model currently exists.

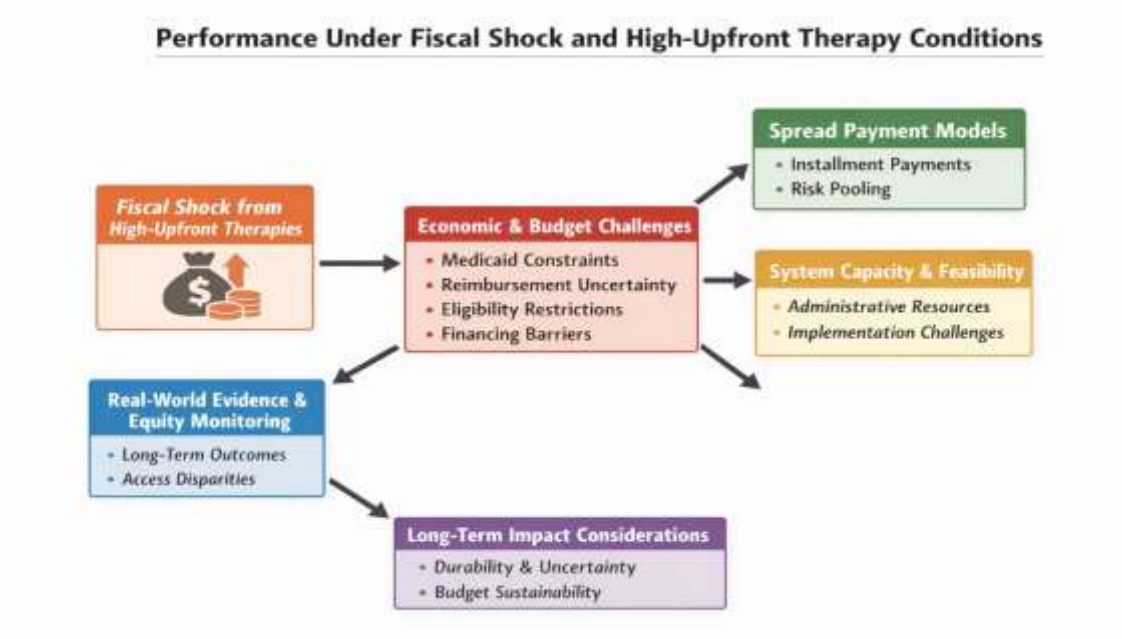
5. Behavioral and Disparity-Focused Models

- **Long-Term Value Estimation: Low.** These frameworks are not designed for economic valuation or clinical outcome projection. Their contribution is in identifying barriers that economic models overlook, not in quantifying therapeutic value.
- **Short-Term Fiscal Forecasting: Low.** Behavioral and equity analyses do not generate expenditure projections. Their value lies in explaining utilization patterns rather than forecasting costs.
- **Equity Sensitivity: Very High.** This is the defining strength of this framework class. Studies consistently demonstrate their capacity to detect racial, ethnic, and income-based disparities in prescribing, adherence, and access that other frameworks miss entirely (Friedman et al., 2021; Kizza, Aduampong, & Kaiser, 2025; Kimerling et al., 2020).
- **Adaptability to Policy Reform: Moderate.** Behavioral frameworks can inform policy design but are typically applied as diagnostic tools rather than adaptive policy models.
- **Administrative Feasibility: High.** Survey-based, qualitative, and claims-stratification approaches used in behavioral and disparity analyses are methodologically accessible and do not require specialized infrastructure.
- **Scalability Across States: High.** The methods are broadly applicable and can be implemented using nationally available survey data (e.g., MEPS, NHIS) or state-level Medicaid claims data.

4.3 Performance Under Fiscal Shock and High-Upfront Therapy Conditions

High-upfront, one-time therapies create fiscal shock that affects economic valuation, financing mechanisms, system governance, and patient access simultaneously. Traditional cost-effectiveness models struggle to accommodate large immediate expenditures within annual Medicaid budgets. Budget and payment structures may generate reimbursement uncertainty and eligibility restrictions, delaying access. Spread-payment arrangements and federal risk-pooling mechanisms improve fiscal stability by distributing costs over time. Implementation feasibility depends on administrative capacity at the system level. Without integrating real-world evidence and equity monitoring, budget impact models may detect spending spikes but fail to capture durability uncertainty and disparities in patient access.

Figure 1 below illustrates these interrelated dynamics.



Adapted from "A New Framework for Quantifying Healthcare Value Using Real-World Evidence," by Pyenson et al., 2024, Milliman.

The model demonstrates sequential dependency: fiscal value must align with payment feasibility, which must align with coverage design, and ultimately translate into equitable patient-level access.

4.4 Adaptability and Real-World Implementation Capacity

RWE-based frameworks demonstrate superior adaptability when policies shift, particularly within Medicare drug negotiation environments (Tunis et al., 2025). Predictive analytics modeling further enhances scenario planning capacity for Medicaid populations (Adetunji, 2025).

Policy evaluation studies of Medicaid accountable care organizations reveal moderate performance improvements in cost containment and quality outcomes, but variable access effects (Holm et al., 2024). Oncology care model evaluations highlight how reimbursement design influences provider participation and high-cost drug utilization (Theroux et al., 2020).

Behavioral frameworks reveal that administrative burden and patient engagement challenges operate independently of reimbursement adequacy (Kimerling et al., 2020). Coverage discontinuities ("coverage cliffs") further illustrate structural barriers unrelated to cost-effectiveness metrics (Whidden, 2022).

Table 2: Robustness Assessment Under Policy Variability

Scenario	Most Robust Framework
State Medicaid Variation	RWE + Policy Analytics
Medicare Cost-Sharing Increases	Behavioral + Spending Analysis
Gene Therapy Launch	Payment Innovation + HTA Recalibration
Cross-Jurisdiction Market Access	Comparative HTA
Regulatory Complexity	Market Access Reviews

Sources: Theroux et al., 2020; Whidden, 2022; Adetunji, 2025

Hybrid evaluation frameworks consistently demonstrate the greatest robustness across policy shifts.

4.5 Comparative Synthesis of Findings

Across both stable (nominal) and high-uncertainty policy environments, such as the introduction of high-cost gene therapies or shifts in Medicare cost-sharing structures, the comparative findings reveal differentiated strengths and weaknesses among methodological frameworks used to evaluate access barriers. Cost-effectiveness analysis (CEA) and traditional health technology assessment (HTA) frameworks consistently perform well in estimating long-term therapeutic value, particularly when modeling lifetime outcomes and quality-adjusted life years, as demonstrated in gene therapy evaluations (Basu et al., 2024). However, standard ICER thresholds and discounting assumptions often require recalibration when applied to one-time curative therapies with high upfront costs and long-term uncertainty (Angelis et al., 2020; Coyle et al., 2020).

Budget impact models, by contrast, dominate short-term fiscal planning and are particularly useful for projecting immediate Medicare and Medicaid expenditure growth associated with specialty drugs (Trish et al., 2021; Conti et al., 2025). Yet these models typically focus on aggregate spending effects and may underperform in identifying inequitable access patterns or cost-related nonadherence (Kizza, Aduampong, & Kaiser, 2025).

The integration of real-world evidence (RWE) significantly enhances adaptability, particularly within Medicare drug price negotiations and post-market reassessment contexts (Tunis et al., 2025; Pyenson et

al., 2024). RWE frameworks improve transparency and allow dynamic evaluation of utilization trends and outcome heterogeneity across beneficiary groups.

Spread-payment mechanisms and innovative insurance redesign proposals effectively mitigate fiscal shock associated with one-time therapies by smoothing costs across time and stakeholders (Michelsen et al., 2020; Conti et al., 2025). However, these models introduce administrative complexity and require robust outcome tracking infrastructure.

Finally, behavioral and disparity-oriented frameworks remain indispensable for identifying racial, ethnic, and income-based inequities in prescribing patterns and treatment adherence (Friedman et al., 2021; Kimerling et al., 2020). These approaches reveal access barriers often invisible within purely fiscal or cost-effectiveness models.

Generally, the evidence indicates that integrated hybrid methodological approaches combining CEA, budget forecasting, RWE, payment reform analysis, and behavioral assessment provide the most balanced and resilient structure for evaluating patient access barriers in Medicaid and Medicare populations.

5.0 DISCUSSION

5.1 Practical Implications for Medicaid and Medicare Policy

The comparative analysis demonstrates that evaluating patient access barriers to high-cost therapies requires more than traditional economic modeling. While cost-effectiveness analysis (CEA) and HTA frameworks remain foundational for assessing long-term therapeutic value, their standalone application is insufficient in public payer environments characterized by fiscal constraints, political oversight, and equity mandates. For example, recalibration of HTA thresholds for gene and cell therapies reflects recognition that curative intent treatments challenge conventional valuation paradigms (Angelis et al., 2020; Coyle et al., 2020). Policymakers within CMS and state Medicaid programs must therefore interpret ICER results within broader fiscal and social contexts.

Short-term fiscal management remains a dominant concern, particularly as specialty drug spending continues to rise in both Medicare and Medicaid populations (Trish et al., 2021). Budget impact analyses provide immediate visibility into expenditure trajectories but may inadvertently incentivize short-term containment strategies that restrict beneficiary access (Conti et al., 2025). This tension underscores the need to align fiscal planning with longitudinal outcome measurement.

Real-world evidence (RWE) frameworks represent a significant methodological advancement in this regard. The integration of claims-based and post-market data into Medicare drug negotiation processes reflects a shift toward adaptive value assessment (Tunis et al., 2025). By incorporating utilization heterogeneity and observed treatment durability, RWE enhances transparency and improves alignment between reimbursement and clinical outcomes (Pyenson et al., 2024). For Medicaid programs, predictive analytics tools further support scenario modeling and forward-looking policy simulations (Adetunji, 2025).

Payment innovation models also carry substantial policy implications. Outcome-based spread payments and federal reinsurance proposals offer mechanisms to mitigate the fiscal shock of one-time therapies while preserving access (Michelsen et al., 2020; Conti et al., 2025). However, successful implementation depends on administrative capacity, standardized outcome measurement, and intergovernmental coordination factors that vary widely across states.

5.2 Trade-Offs Among Methodological Frameworks

The findings reveal several fundamental trade-offs that shape methodological suitability.

1. **Long-Term Value vs. Short-Term Fiscal Stability** CEA models emphasize lifetime value but may conflict with annual Medicaid budget cycles. Budget impact models prioritize immediate fiscal stability but risk undervaluing durable clinical benefits (Basu et al., 2024; Trish et al., 2021).
2. **Analytical Precision vs. Administrative Feasibility** RWE integration and spread-payment mechanisms provide nuanced, adaptive evaluation capacity but require significant data infrastructure and administrative oversight (Tunis et al., 2025; Michelsen et al., 2020). Simpler fiscal models are easier to implement but less comprehensive.
3. **Fiscal Metrics vs. Equity Detection** Spending projections and ICER thresholds often obscure inequitable prescribing patterns and cost-related nonadherence (Friedman et al., 2021; Kizza, Aduampong, & Kaiser, 2025). Behavioral frameworks reveal patient-level burdens that purely economic models fail to capture (Kimerling et al., 2020).
4. **Standardization vs. Contextual Flexibility** Cross-jurisdiction variation in market access decisions demonstrates that standardized evaluation approaches may not translate uniformly across Medicaid programs (Tunis et al., 2021). Local policy conditions significantly influence implementation feasibility.

5.3 Equity and Structural Access Considerations

One of the most consistent findings across the reviewed literature is that financial coverage alone does not guarantee equitable access. Racial, ethnic, and income disparities in medication prescribing patterns among Medicare beneficiaries illustrate systemic inequities that persist despite nominal coverage (Friedman et al., 2021). Cost-related nonadherence further demonstrates that out-of-pocket exposure influences treatment continuation (Kizza, Aduampong, & Kaiser, 2025).

Coverage discontinuities and administrative "cliffs" can also disrupt access pathways, even when therapies are deemed cost-effective (Whidden, 2022). Moreover, variability in state Medicaid coverage policies for gene and cell therapies produces uneven national access patterns (Allen et al., 2023; Auletta et al., 2023). These findings reinforce the necessity of embedding equity metrics within methodological evaluation frameworks rather than treating them as secondary considerations.

5.4 Toward an Integrated Hybrid Evaluation Model

The comparative findings support the adoption of integrated hybrid methodological approaches that combine:

- Economic valuation (CEA/HTA) for long-term benefit estimation
- Budget impact modeling for fiscal feasibility assessment
- RWE integration for adaptive reassessment
- Payment innovation analysis for fiscal smoothing
- Behavioral and disparity analytics for equity evaluation

Such hybridization enhances robustness across policy environments characterized by uncertainty, fiscal volatility, and demographic diversity. Policy evaluation studies of Medicaid accountable care organizations and oncology care models demonstrate that payment structure design influences both cost and utilization patterns. Therefore, access evaluation must extend beyond therapy pricing to encompass delivery system incentives and performance measurement alignment.

5.5 Future Research and Policy Directions

To bridge current methodological gaps and enhance equitable access, several targeted priorities emerge for advancing frameworks in Medicaid and Medicare policy. Future research and policy should prioritize: (1) standardizing equity metrics in economic models through disparity-adjusted outcomes and stratified

RWE; (2) developing longitudinal outcome infrastructure via CMS-state-manufacturer data sharing for outcome-based payments; (3) simplifying administration in spread-payment models to avoid new barriers; and (4) harmonizing Medicaid coverage criteria across states to enhance national equity in high-cost therapy access.

6.0 CONCLUSION

This study underscores the multifaceted nature of evaluating patient access barriers to high-cost therapies within Medicaid and Medicare populations. No single methodological framework adequately captures the combined challenges of therapeutic value assessment, fiscal sustainability, policy implementation, and patient-level equity. Cost-effectiveness analysis and health technology assessment provide strong insight into long-term clinical and economic value, while budget impact models are more effective in addressing short-term fiscal pressures faced by public insurance programs. However, these traditional approaches often overlook behavioral dynamics and disparities in treatment access. The integration of real-world evidence improves adaptability and supports continuous reassessment of treatment outcomes and utilization patterns. Innovative payment models such as spread-payment arrangements can help mitigate fiscal shock associated with high-upfront therapies, though their implementation demands administrative infrastructure that varies considerably across state Medicaid systems.

The comparative analysis and illustrative application presented in this study demonstrate that integrated hybrid methodological approaches, combining economic evaluation, fiscal forecasting, real-world evidence, payment innovation, and equity assessment within a phased and iterative structure, offer the most comprehensive and sustainable framework for evaluating access barriers. Critically, the inclusion of equity monitoring as a continuous feedback mechanism, rather than a secondary consideration, distinguishes this integrated model from conventional evaluation practices.

As high-cost therapies continue to proliferate and public insurance enrollment expands, the consequences of methodological fragmentation will grow more severe. Policymakers who rely on cost-effectiveness analysis alone risk approving therapies that remain inaccessible to the populations who need them most. Those who rely solely on budget impact models risk restricting access to treatments with substantial long-term value. An integrated approach does not eliminate these tensions, but it ensures they are identified, measured, and addressed across interconnected evaluation phases. The goal is not simply to determine whether a therapy is worth funding, but to ensure that funding decisions translate into equitable access at the patient level. Without that integration, the promise of transformative therapies will remain unevenly fulfilled across the communities that Medicaid and Medicare are designed to serve.

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