Balancing Specialty Drug Pricing and Innovation – A Framework for Sustainable Access and Affordability in the U.S. Healthcare System

DR. RICHARD AMISSAH MD/MBA
Vanderbilt University

Abstract- ***The U.S. healthcare system is facing a serious and unsustainable dilemma between the two-fold purpose of innovation in specialty drugs as an incentive and affordable and equitable access to patients. High prices caused by expensive biologic drugs and transformative cell and gene therapy and present significant access barriers to patients threaten the financial stability of the payers. The following discussion is a summary of a detailed analysis of such sources as IQVIA, the Centers of Medicare and Medicaid Services (CMS), and the Assistant Secretary of Planning and Evaluation (ASPE) to quantify the spending trends, the budget implications, and the multi-faceted character of the correlation between the price, innovation, and accessibility to patients. Two new inter-related systems have to be used to address this systemic issue; a multi-attribute Value-Sustainability Index (VSI) to capture comprehensively the therapeutic, innovative and systemic value of a drug and a National Affordability Threshold (NAT) to provide a base to price debate on macroeconomic reality. These quantitative figures indicate that the increase in the amount of Medicare Part D gross expenditure by 566.5 percent in 2012-2021 resulted in an economic amount exceeding 50 percent of the spending on specialty drugs, with much of that highly concentrated within the top 1 percent of the drugs which have caused over 41 percent of Part D spending.2 Discussion The author concludes the manuscript by pleading to implement the VSI and NAT frameworks through a multi-stakeholder and voluntary Sustainable Access Compact. This small would assist in introducing value-consistent payment models, such as outcomes-based contracting and indication-based pricing, in order to create a predictable and long-term market that does not merely compensate the actual innovation but also brings transformative medicines to the patients that need them.***

I. INTRODUCTION

The Unsustainable Tension in U.S. Specialty Medicine

The business model of Biopharmaceutical market development in the United States is paradoxical: it is the strongest engine of medical innovation in the world, but it is designed to systematically destroy the value of its own innovations on the society.5 But these very prices pose a strain to the budgets of both the government and the non-government, cost patients debilitating out-of-pocket costs and in the long term result in an unsustainable direction of all those involved in the system.4

The emergence of specialty drugs is in the center of this challenge. Although there is no single, standardized definition, a functional consensus on the characteristics of a drug has developed around a set of attributes that tend to make the drug expensive; it is usually administered, handled, or stored in a special manner (such as a refrigerated or cold chain); and is usually distributed through a restrictive number of specialty pharmacies offering high-intensity patient monitoring and support, making the economics more complex.10

This is an active market that is functioning in an era of radical policy change. A watershed event in pharmaceutical economic lifecycle The Inflation Reduction Act (IRA) of 2022 gives the federal government, on a case by case basis, the authority to negotiate the price of certain high-expenditure drugs reimbursed by Medicare, a novel authority in the history of the pharmaceutical industry that instills new powerful incentives neighboring the R&D strategies.18 The IRA subjects small-molecule drugs (typically pills) to negotiation after nine years post-approval, versus thirteen years of large-molecule

Introduction in a low population of needy orphans begins the nine-year clock, which can jeopardize the revenue of a subsequent, larger indication.18 This cost containment policy can inadvertently drive investment away to more easily administration oral pharmaceuticals and dishearten the same post-approval research that has historically extended the therapeutic utility of existing medicines.19

The intensity of the specialty drug market indicates the acuity of these concerns. The IQVIA and Assistant Secretary for Planning and Evaluation (ASPE) data reveal that although the specialty medicines dominate a fraction of the total prescription, they already occupy more than half of the pharmaceutical expenditure in the United States, and ideally this trend is expected to increase to over half of the total expenditure in the developed markets in 2028.23 This report aims to go beyond diagnosis of this well-documented issue, to an actual proposal of a tangible, evidence-based structure to reform. It proposes a new design of the balance between innovation and affordability by quantifying the trends, analyzing the incentives of stakeholders, and learning lessons following the global experience in order to provide a sustainable and fair future of American healthcare.

The Evidence Base: Quantifying the Trajectory of Specialty Drug Spending and Impact

A rigorous analysis of spending data from the past decade reveals a market undergoing a structural transformation, characterized by the rapid fiscal dominance of specialty drugs, the concentration of spending on a small number of products, and the emergence of ultra-high-cost curative therapies that challenge the foundations of traditional reimbursement models.

Unprecedented Growth and Spending Concentration

The increase in the trend of spending on specialty drugs has been insatiable and it has essentially transformed budgets of payers. In the United States, specialty drug spending is expected to grow 43% to 301billion by 2021, and to 50% of total pharmaceutical spending by 2028, due to advances in oncology and immunology, and IQVIA projects that specialty medicines will be spending 43% of the world and more than 55% of major developed market spending by 2028.22 Total U.S. spending on specialty drugs has increased by 43% to 301billion

This is more apparent in the public payer programs. In Part D, the gross expenditure on specialty drugs in the period between 2012 and 2021 more than doubled by 566.5 percent with a compound annual growth rate (CAGR) of 23.5, contributing to the total Part D spending on specialty drugs that shot up by over 71.1 percent over the same time frame.25

This was a boom that is followed by a severe concentration of expenditure. Under Medicare Part D, the 1% of highest-spending drugs comprised 41.1% of total spending in 2021, which is a sharp rise in this measure over 31.4% of total spending in 20122 This growing concentration is not only a concentration around a small group of high-price drugs, but also a marketplace still with limited competition. ASPE-based data show that 81 percent of biological products had but one manufacturer and that these monopolistic products had 74 percent of the total biologic expenditure, making the market environment where conventional competitive forces on price are inapplicable.26

Figure 1: The Rise of Specialty Drugs in U.S. Pharmaceutical Spending, 2016–2025 (Projected)

Year	Total Specialty Spending (USD Billions)	Total Non-Specialty Spending (USD Billions)	Specialty Share of Total Spending (%)
2016	$211	$309	40.6%
2017	$225	$294	43.4%
2018	$245	$295	45.4%
2019	$268	$302	47.0%
2020	$287	$303	48.6%
2021	$301	$302	49.9%

2022	$328 (proj.)	$305 (proj.)	51.8%
2023	$359 (proj.)	$308 (proj.)	53.8%
2024	$395 (proj.)	$310 (proj.)	56.0%
2025	$434 (proj.)	$312 (proj.)	58.1%

Note: Data for 2016-2021 synthesized from ASPE.[22] Projections for 2022-2025 are illustrative, based on growth trends identified in IQVIA reports.[23]

The Budgetary Shock of Curative Therapies

Although the current spending growth has been precipitated by high cost chronic therapy, the introduction of potentially curative cell and gene therapies (CGTs) has thrown a different and unique challenge on financing the health system. These therapies that have an initial list price of a few million dollars per patient interfere with payment models of incremental, chronic care.27

The present expenditure on CGTs is low though on an exponential rate and is estimated to be 5.9 billion in 2023, 38% higher than the 2022.28. It would influence the budgetary takeaway in a very large way. Different simulation models project that soon the annual U.S. expenditure on new gene therapies is likely to reach $20 billion to $25 billion 3, with a cumulative expenditure impact of more than 300 billion by 2034.29 One-time, expensive nature of novel gene therapies offers extreme budget volatility and actuarial risk that may present a moderate or significant burden to smaller employers, regional health plans, and state Medicaid programs that simply do not have the scale to absorb such expenditures.

This dilemma is a basic time conflict in the financial model of healthcare. The cost of a curative therapy in the first year is multi-million dollars, which is paid by the payer, whereas the savings of long-term care are distributed over the lifetime of the patient, which means that the initial payer bears the entire financial risk, and the benefits of the long-term care are incurred by the subsequent payer. This lack of incentive creation causes a market failure that will demoralize coverage of curative therapies even those with a net long term value to the overall healthcare system.

Price-Innovation-Access Nexus

The reasoning behind the high cost of specialty drugs is based on the notion that it is required to encourage R&D that is risky.6 It has however been indicated that there is the increasing disparity between cost, therapeutic benefit and patient access. New drugs are always priced at record-breaking rates; the median annual price of new drugs approved in 2023 was 300,000 which was 35 percent higher than the previous year.32 These prices tend to be higher than conventional value-for-money levels employed by health technology assessment agencies in other countries.

Importantly, these expensive prices and its implies cost-sharing result in putting a serious obstacle on patient access, which casts doubt on the effectiveness of the therapies in practice. Research is consistent that higher patient cost-sharing on specialty drugs is correlated with lower medication adherence.33 National survey studies have shown that almost a third of new prescriptions to oral cancer, even when covered by Part D, are not taken by patients because of its high cost.35 The most inventive drug does not matter when the patient is not able to get it.

Table 2: Concentration of U.S. Payer Spending on Top Specialty Drugs, 2024

Drug Name	Manufacturer(s)	Primary Indication(s)	Total Gross Medicare Part D Spend (Jun 2022-May 2023)	Number of Medicare Part D Users
Januvia	Merck	Type 2 Diabetes	$4.09 billion	850,000+
Eliquis	Bristol Myers Squibb / Pfizer	Blood Clot Prevention	$12.5+ billion (est.)	2,000,000+
Xarelto	Janssen (J&J)	Blood Clot Prevention	$6.0+ billion (est.)	1,000,000+
Entresto	Novartis	Heart Failure	$2.88 billion	500,000+
Imbruvica	Pharmacyclics (AbbVie) / Janssen (J&J)	Leukemia, Lymphoma	$2.66 billion	40,000+
Jardiance	Boehringer Ingelheim / Eli Lilly	Type 2 Diabetes, Heart Failure	$7.0+ billion (est.)	1,300,000+
Stelara	Janssen (J&J)	Psoriasis, Crohn's Disease	$3.0+ billion (est.)	60,000+
Farxiga	AstraZeneca	Type 2 Diabetes, Heart Failure	$3.0+ billion (est.)	700,000+
Ozempic	Novo Nordisk	Type 2 Diabetes	$10.0+ billion (est.)	1,500,000+
Enbrel	Amgen	Rheumatoid Arthritis, Psoriasis	$2.5+ billion (est.)	40,000+

Note: Data synthesized from CMS Drug Spending Dashboards and KFF analyses of drugs selected for IRA negotiation.[36] Spending figures are gross and may vary based on reporting period; user counts are approximate. This table highlights drugs frequently appearing in top-spending lists, many of which are specialty or have specialty-tier placement.

Proposed Frameworks for Realigning Value and Sustainability

To address the systemic misalignments identified, a new conceptual architecture is required—one that moves beyond the current adversarial pricing environment toward a structured, evidence-based approach to defining and rewarding value. This report proposes two complementary frameworks: the Value-Sustainability Index (VSI) to provide a holistic assessment of a drug's worth, and a National Affordability Threshold (NAT) to ensure that pricing

decisions are grounded in the economic capacity of the healthcare system.

The Value-Sustainability Index (VSI): A Multi-Attribute Framework for Assessment

The VSI is constructed to provide a more subtle and detailed analysis of a novel specialty drug than can be done through single-metric methods such as cost-per-Quality-Adjusted-Life-Year (QALY). It is a composite index which combines three different but equally important areas of value to give a cumulative score which can be used to inform negotiations and coverage choices.

Component 1: Therapeutic Value. This field determines the vital clinical value drug brings to the patients. Measures would be based on comparative effectiveness studies, and would be in terms of improvements in overall survival, progression-free survival, quality of life, and patient-reported outcomes relative to the current standard of care.40 This aspect would also aim to explicitly weight the condition under treatment severity and the level of unmet medical need, to make sure that drugs to devastating diseases without alternatives are identified as high-value drugs in society.

Component 2: Innovation Novelty. This measure is used to measure how scientifically advanced a product is and thus, rewards real breakthrough over a more incremental or a me-too drug. Among the relevant metrics would be novelty of the mechanism of action, first-in-class status, such designations as the FDA Breakthrough Therapy designation, and the R&D intensity, including investment as a percentage of revenue, and ability of a new product to be a platform technology (e.g. an mRNA or CRISPR platform).40

Component 3: Sustainability of system. This area directly responds to the economic consideration of a new therapy on the healthcare system which is of paramount importance to payers.31 Metrics would involve a formal budget impact analysis, which estimates the overall cost to the payer depending on price of the drug and the population size of the patients eligible. It would also consider the possible downstream cost savings like fewer hospitalizations or avoidance of other health services.40 Lastly, this aspect would also consider the manufacturing and supply chain resiliency factors because the value of a drug would not be as high when it is frequently affected by shortages.44

The combination of the three of these domains would produce a multidimensional score (e.g., out of 1-100) which is transparent. Subsequently, this score would act as a standardized, evidence-based baseline through which manufacturers and payers can discuss the matter and take the issue of price negotiations out of context in favor of a more comprehensive discussion of the value and role of a drug as a whole.

(Description of the Conceptual diagram: The central circle is the Value-Sustainability Index (VSI) Score, which is being fed by three other circles around it. The former, "Therapeutic Value," features such metrics as "Comparative Effectiveness," "Unmet Medical Need," and "Patient-Reported Outcomes." The second is Innovation Novelty gives first-in-class status, R&D Intensity and Platform Technology. The third is the "System Sustainability," which includes such items as the "Budget Impact Analysis," the "Total Cost of Care Offset," and the Supply Chain Resilience).

A National Affordability Threshold (NAT): Defining the Limits of System Capacity

The VSI also provides the answer to the question, Is this drug valuable? but the NAT is designed to provide a response to the equally important question, Can our system afford it at this price? The U.S. does not have a clear, system-wide affordability standard, which is one of the essential characteristics of most other OECD healthcare systems that can be used to make a rational decision on pricing negotiations.45 NAT would occupy this void.

The NAT would not be a fixed price limit but would be a dynamic and evidence-based price limit on the highest impact that a new therapy entering the market has on the aggregate budget in a particular year. It would be calculated basing on the macroeconomic indicators, e.g., the anticipated increase in national health spending, the growth in the GDP, and the opportunity cost of healthcare spending (i.e., the worth of alternative health services which would get displaced by covering the cost of a new drug).7

The implementation of the NAT would be its use as a policy trigger. When it is determined that a new drug, even with high VSI score, has an estimated first-year change in budget that surpasses the NAT, then it would automatically result in a necessity that manufacturers and payers would engage in non-traditional payment methods. These may involve an outcome-based contracting, an indication based pricing or gradual rollout of the same to control its budgetary shock in the long run. This would send a very explicit, certain message to the manufacturers about the capacity of the system to absorb high-cost innovations and oblige a proactive and cooperative effort in dealing with the issue of affordability instead of a reactive response, based on access limitations in retrospect.

The VSI and NAT are very effective as a two-part test. They rebrand the negotiation as an adversarial bilateral price haggle to a structured and evidence-based conversation both on value and social affordability. This framework enables the payers to have rational and defensible decision making instruments and gives the manufacturers a clear and predictable framework through which they can prove the value of their innovation.

Discussion: Stakeholder Implications and Global Context

The suggested system of VSI and NAT would essentially change the strategic environment of all key players in the healthcare system of the U.S. These tools would transform incentives and reduce distortions induced by policies by establishing a clear and evidence-based architecture of value evaluation and price negotiation that would put the U.S. into better alignment with global practices in the management of pharmaceutical spending.

New Paradigm to the U.S. Stakeholder

To the Manufacturers: A strategic change in investment in R&D would be the key effect of this: it would no longer focus on producing incremental, or even me-too drugs, but rather on establishing a truly transformative therapy that could be used to negotiate high VSI scores.5 A high score in Therapeutic Value and Novelty in Innovation would become an important tool in pricing and market access negotiations. This is a very strong counter-narrative to the existing system where high-priced launches do not necessarily correspond to high value. Besides, the frameworks would add a certain predictability and transparency to the pricing process, decreasing the uncertainty that the market entry is currently dominated by.46 A clear definition of how the value is created (VSI) and what the machine is capable of (NAT) would enable the rational business planning. Such structures may also serve to alleviate certain ill effects of negative incentives that the IRA has developed. Indicatively, a small-molecule drug very innovative with a high VSI score may have more attractive negotiation conditions that partially compensate the disincentive of the shorter nine-year window before the innovation can be negotiated.18

In the case of the Payers and Health Systems: These parties would be the most direct beneficiaries, as the VSI and NAT would give them a strong, defendable platform to push back against prices that are not correctly aligned with value, or are simply untenable.32 The current landscape also makes it seem that U.S. payers are increasingly vulnerable, with limited means to effectively negotiate, particularly as compared to their foreign counterparts.47

To Policymakers: The VSI and NAT can provide a more sophisticated and sustainable policy framework, versus the current drug selection procedure of the IRA, which relies mostly on overall spending.36 CMS could use these frameworks to form its negotiation policy, establishing a model that would be voluntarily adopted by the business community. It would provide a unified and nationally based criterion of value determination, which would bring the U.S. a step closer to the systematic methodology of health technology assessment (HTA) bodies in other developed countries.

To Patients: This reformed system is aimed at enhancing patient outcomes and access in the end. The frameworks will enable more patients who have the most to gain to access high-value drugs by making the price more reflective of the value and ensuring the system is sustainable, a primary cause of medication non-adherence and prescription abandonment.

Comparison with OECD Models

The necessity of such a system is supported by the fact that the U.S. is an outlier in the pharmaceutical pricing in the world. The statistics constantly indicate that the U.S prices of brand-name drugs are not merely three times greater than the average of 33 other OECD nations but are in fact supported by a market system which allows prices that are well beyond what they have shown to be worth.50

Value-based pricing is not a new concept in many European nations, where typically a national HTA body will undertake a strict measure of the additional benefit of a new drug over an existing standard of treatment and that measurement is the foundation of price negotiation.45 The proposed VSI framework also applies the same concept of comparative effectiveness, however, with the addition of novelty of innovation and system sustainability as a definition of value.

But the experience of the European has its own lessons. Other policies of national cost-containment, such as external reference pricing (setting prices based on prices in other countries) and budget caps with compulsory clawbacks are likely to dilute the relationship between a formal value evaluation and the final negotiated price, which is the goal of a value-based system.45 The proposed U.S. model, by directly linking the VSI and NAT to particular paths to payments and access, would establish a more direct and transparent relationship between value and price. The frameworks have the potential to overcome the long-established lack of trust between payers and manufacturers by establishing a common currency of value (evidence-based) to transform the process of negotiations into a collaborative one aimed at ensuring access to valuable medicines on a sustainable basis.

Actionable Policy Recommendations for a Sustainable Future

Translating the VSI and NAT frameworks from concept to reality requires a clear implementation pathway. This involves embedding them within innovative payment models and fostering a new, collaborative relationship among stakeholders through a national compact.

Implementing Value-Aligned Payment Models

The VSI and NAT are not an isolated instrument; they are created to inform the choice and arrangement of purposeful, value-based compensation patterns that can be executed founded on a drug-certain profile.

Outcomes-Based Contracting (OBCs): In treatments with high VSI scores and much uncertainty about how they actually perform in the real world, like with first in class biologics or gene therapies, OBCs are the model of choice. In such arrangements, the payment is directly related to the accomplishment of a pre-established clinical or economic result in a particular population of patients.52 This moves some part of the risk of performance off the payer and on to the manufacturer. The NAT may have a direct launch: when the projected budget implication of a drug surpasses the threshold, an OBC would become the default way to market access.54

Indication-Based Pricing (IBP): In oncology, where one drug can produce transformative benefits to a certain type of cancer but only marginal benefits to another, IBP would play a crucial role in establishing prices based on each individual indication.55 The VSI would be computed on each individual indication, and the scores would direct the coverage of indication-specific prices or rebates. Operationally difficult, but necessitating advanced data systems to monitor utilization by diagnosis, PBMs such as Express Scripts and CVS Caremark have already started to adopt IBP in specific classes of drugs, already proving feasible.56

Tiered Launch Sequencing: Policy can encourage manufacturers to early launch a novel drug in the indication with the most patients to receive the greatest early returns, which may not be the indication in which the novel drug delivers the greatest value, or the one with the greatest unmet need.59 Policy would encourage manufacturers to early launch a new drug in the indication with the largest number of patients to maximize early returns, which is not necessarily the indication where the novel drug delivers the greatest benefit or which has the greatest need.

Building a National Healthy Access Compact

Taking into consideration the political and practical intricacies of the U.S. healthcare structure, a top-down

governmentally imposed adoption of these structures would tend to experience substantial legal and political issues. The development of a voluntary, multi-stakeholder, Sustainable Access Compact is a more reasonable way to go. This would be a formal contract between major payers, manufacturers, PBMs, and health systems based on collaborative efforts such as Oregon Value-Based Payment Compact 60 but with government agencies, such as CMS assuming the role of conveners and participants.

Commitments: Signatories to the Compact would undertake to utilize the VSI and NAT as the generic, generalized models of all price and access bargains of novel specialty drugs. The benefits of this so-called grand bargain would be that manufacturers would have a predictable route to the market and favorable access to those drugs that prove to be of high value, and payers would have budget sustainability and avoid unsustainable price surges.

Governance: The Compact would be supervised by an independent, multi-stakeholder governance body, which is neutral. Such a body would take care of the maintenance and updating of the VSI and NAT methodologies keeping them up to date with the current scientific findings and the economic state of affairs. It would also play a dispute resolution role and best practice exchange, which is similar to other countries through an HTA body.

This is a more practical approach to collaborating that is more politically feasible. The absence of a common data infrastructure to monitor patients and outcomes is a significant barrier to the higher-order models of payment, a significant factor identified by both payers and manufacturers as an obstacle to the implementation of the Compact.62 The Compact can be a pre-competitive consortium to fund and develop the necessary infrastructure that would solve a collective action problem that cannot be successfully addressed by one body. In such a manner, the Compact would be transformed into a potent tool to create the market capabilities needed to construct an actual value-based healthcare system.

Table 3: Comparison of U.S. and Select OECD Value Assessment and Pricing Frameworks

Feature	U.S. (Current)	U.S. (Proposed VSI/NAT)	Germany (AMNOG)	United Kingdom (NICE)
Primary Value Metric	No standard metric; varies by payer.	VSI (multi-attribute: clinical, innovation, systemic).	Added therapeutic benefit vs. comparator.	Cost per QALY.
Use of Explicit Threshold	No.	Yes (NAT for budget impact).	No explicit cost-effectiveness threshold.	Yes (explicit QALY threshold, ~$25,000-$38,000).
Link to Pricing	Opaque negotiation; influenced by list price and rebates.	VSI score and NAT status directly inform price and trigger specific payment models.	Benefit rating determines negotiation power for a rebate off the launch price.	Cost-effectiveness determines recommendation for coverage at a given price.
Key Strength	Market-based flexibility; rapid access for approved	Balances multiple value domains; explicitly incorporates	Rigorous comparative effectiveness	Transparent, evidence-based process with clear thresholds.

	drugs (if affordable).	affordability.	assessment.	
Key Weakness	High prices, inequitable access, lack of transparency.	Requires multi-stakeholder buy-in and data infrastructure.	Does not formally consider cost-effectiveness in initial benefit rating.	Can be slow; may undervalue innovation for rare diseases or curative therapies.

Note: Information synthesized from analyses of international pricing systems.[45] QALY threshold in UK is approximate and subject to modifiers.

Conclusion: Safeguarding Innovation and Patient Access

The market of specialty pharmaceuticals in the U.S. is at the crossroad. The present trend of increasing prices and rising access barriers is unsustainable to patients, payers, and the health of the innovation ecosystem in the long-term. Policymakers and healthcare leaders do not have a one-or-the-other option between encouraging innovation and making healthcare affordable. Instead, it is a decision on whether to continue with the dysfunctional status quo or adopt the new paradigm wherein incentives are aligned strategically around the value, sustainability, and patient outcomes.

The models suggested in this paper, the Value-Sustainability Index and the National Affordability Threshold, provide an obvious course of action and head in a new direction. They are not aimed at smothering the innovation process, but to direct it to its most fruitful purposes by stimulating real therapeutic breakthroughs and scientific innovation. These tools can turn adversarial negotiations into collaborative alliances by developing an open and evidence-based language of defining and measuring value. Their direct inclusion of a measure of system-wide affordability will make the innovation market sustainable even to future generations.

A voluntary Sustainable Access Compact as a way of operationalizing these frameworks is a realistic solution to reform in the complicated American situation. It appreciates that lasting change should not only be a good policy design but also buy-in and active involvement of every stakeholder. The trust and the collective infrastructure it can establish is what it takes to deploy more complex payment models such as results-based contracting and indication-based pricing on a large scale.

After all, in order to secure the future of pharmaceutical innovation, it is important to make sure that its fruits are made available to the society. A system which creates cures that are unaffordable to the patients is a system which has failed in its role as a first line mission. With price and value and the market re-established based on the principles of sustainability, we will be able to create a healthcare system that not only propels medical research but also fulfills its purpose of enhancing the health of humans. The era of moderate modifications is long gone, a radical re-design of the market is needed to ensure the future of American innovation and patient health.

REFERENCES

[1] Happe, L. E., Niu, S., & Svensson, M. (2024). *Concentration of spending and share of specialty drug spending in Medicare Part D over a 10-year period. Journal of Managed Care & Specialty Pharmacy, 30*(12), 1355–1363. https://doi.org/10.18553/jmcp.2024.30.12.1355 Retrieved from https://www.jmcp.org/doi/abs/10.18553/jmcp.2024.30.12.1355

[2] Centers for Medicare & Medicaid Services (CMS). (2024). *Concentration of spending and share of specialty drug spending in Medicare Part D.* Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC11607209/

[3] Doshi, J. A., et al. (2024). *The estimated annual financial impact of gene therapy in the United States. American Journal of Managed Care, 30*(5), e110–e118. https://doi.org/10.37765/ajmc.2024.88435 Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10678302/

[4] Brookings Institution. (2024). *Addressing the trade-off between lower drug prices and incentives for pharmaceutical innovation.* Retrieved from https://www.brookings.edu/articles/addressing-the-trade-off-between-lower-drug-prices-and-incentives-for-pharmaceutical-innovation/

[5] Vogler, S., Paris, V., & Ferrario, A. (2019). *The price of innovation: The role of drug pricing in financing pharmaceutical innovation – A conceptual framework. Journal of Pharmaceutical Policy and Practice, 12*(1), 15. https://doi.org/10.1080/20016689.2019.1583536 Retrieved from https://www.tandfonline.com/doi/full/10.1080/20016689.2019.1583536

[6] Cohen, J. P., & Han, G. (2024). *The price of progress: Understanding innovation and affordability of prescription drugs. American Journal of Managed Care.* Retrieved from https://www.ajmc.com/view/the-price-of-progress-understanding-innovation-and-affordability-of-prescription-drugs

[7] Mendoza, Y. L. M., & Baranauskas, M. C. C. (2024). *Escalating costs of innovative medicines: Perspectives and proposals. Frontiers in Public Health, 12*, 1449707. https://doi.org/10.3389/fpubh.2024.1449707 Retrieved from https://www.frontiersin.org/articles/10.3389/fpubh.2024.1449707/full

[8] Krumholz, H. M., & Anderson, J. L. (2019). *Challenges and potential improvements to patient access to pharmaceuticals. Circulation, 139*(21), e841–e850. https://doi.org/10.1161/CIRCULATIONAHA.119.044976 Retrieved from https://www.ahajournals.org/doi/10.1161/CIRCULATIONAHA.119.044976

[9] Godman, B., et al. (2020). *Pricing of pharmaceuticals is becoming a major challenge for health systems. BMJ, 368,* l4627. https://doi.org/10.1136/bmj.l4627 Retrieved from https://www.bmj.com/content/368/bmj.l4627

[10] Health Affairs Forefront. (2024). *What makes a drug special?: Understanding PBMs' pharmacy definitions.* Retrieved from https://www.healthaffairs.org/content/forefront/makes-drug-special-understanding-pbms-pharmacy-definitions

[11] Office of Inspector General (U.S. Department of Health & Human Services). (2024). *Specialty drug coverage and reimbursement in Medicaid.* Retrieved from https://oig.hhs.gov/reports-and-publications/workplan/summary/wp-summary-0000255.asp

[12] Wikipedia contributors. (2024). *Specialty drugs in the United States. Wikipedia.* Retrieved from https://en.wikipedia.org/wiki/Specialty_drugs_in_the_United_States

[13] Academy of Managed Care Pharmacy (AMCP). (2024). *Specialty pharmaceuticals.* Retrieved from https://www.amcp.org/concepts-managed-care-pharmacy/specialty-pharmaceuticals

[14] Iowa Department of Administrative Services. (2021). *Specialty drugs policy guidance.* Retrieved from https://das.iowa.gov/media/2021/download

[15] The Commonwealth Fund. (2023). *340B drug pricing program: How it works and why it's controversial.* Retrieved from https://www.commonwealthfund.org/publications/explainer/2023/340b-drug-pricing-program-how-it-works-and-why-its-controversial

[16] Kaiser Family Foundation (KFF). (2023). *Explaining the prescription drug provisions in the Inflation Reduction Act.* Retrieved from https://www.kff.org/medicare/explaining-the-prescription-drug-provisions-in-the-inflation-reduction-act

[17] The Hospitalist. (2024). *Understanding the Inflation Reduction Act's impact on prescription drug costs.* Retrieved from https://www.the-hospitalist.org/hospitalist/article/38496/critical-care/understanding-the-inflation-reduction-acts

[18] IQVIA Institute. (2024, September). *The impact of the Inflation Reduction Act on the economic lifecycle of a pharmaceutical brand.* Retrieved from https://www.iqvia.com/locations/united-states/blogs/2024/09/impact-of-the-inflation-reduction-act

[19] PhRMA (Pharmaceutical Research and Manufacturers of America). (2024). *Inflation Reduction Act policy issues.* Retrieved from https://phrma.org/policy-issues/government-price-setting/inflation-reduction-act

[20] Kaiser Family Foundation (KFF). (2024). *How will the prescription drug provisions in the Inflation Reduction Act affect Medicare beneficiaries?* Retrieved from https://www.kff.org/medicare/how-will-the-prescription-drug-provisions-in-the-inflation-reduction-act-affect-medicare-beneficiaries

[21] University of Pennsylvania LDI (Leonard Davis Institute). (2024). *What does the Inflation Reduction Act really mean for drug pricing?* Retrieved from https://ldi.upenn.edu/our-work/research-updates/what-does-the-inflation-reduction-act-really-mean-for-drug-pricing

[22] Cooke, C. E., & Lee, H. Y. (2014). *Cost management through care management, part 2: The importance of managing specialty drug utilization in the medical benefit. American Journal of Managed Care*, *20*(4), e130–e136. Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC4031694/

[23] Pharma Manufacturing. (2024). *Innovation in quality metrics.* Retrieved from https://www.pharmamanufacturing.com/home/article/11315900/innovation-in-quality-metrics

[24] European Federation of Pharmaceutical Industries and Associations (EFPIA). (2024). *A value-based approach to pricing – An EFPIA position paper.* Retrieved from https://www.efpia.eu/media/677284/a-value-based-approach-to-pricing-2.pdf

[25] Hartung, D. M., & Johnston, K. A. (2023). *Specialty drug price trends in the federal 340B drug discount program. Health Services Research and Managerial Epidemiology, 10*(1), 2333392823112327. https://doi.org/10.1177/2333392823112327 Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10398214/

[26] Mulcahy, A. W., et al. (2023). *Understanding payer perspectives on value in the use of pharmaceuticals in the United States. Health Affairs*, *42*(6), 1059-1069. https://doi.org/10.1377/hlthaff.2023.00021 Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10397793/

[27] Khandelwal, N., & Chambers, J. D. (2023). *The current status of outcomes-based contracting for manufacturers and payers: An AMCP membership survey. Journal of Managed Care & Specialty Pharmacy, 29*(5), 463–472. https://doi.org/10.18553/jmcp.2023.29.5.463 Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10397909/

[28] Commonwealth Fund. (2024). *Medicare drug price negotiations: All you need to know.* Retrieved from https://www.commonwealthfund.org/publications/explainer/2024/may/medicare-drug-price-negotiations-all-you-need-know

[29] Office of the Assistant Secretary for Planning and Evaluation (ASPE). (2023). *International prescription drug price comparisons: Estimates using 2022 data.* Retrieved from https://aspe.hhs.gov/sites/default/files/documents/8e057b0a094e6f9b9d01171fce6698f4/international-price-comparisons.pdf

[30] Office of the Assistant Secretary for Planning and Evaluation (ASPE). (2023). *International prescription drug price comparisons: Estimates using 2022 data.* Retrieved from https://aspe.hhs.gov/sites/default/files/documents/277371265a705c356c968977e87446ae/international-price-comparisons.pdf

[31] Carlson, J. J., & Sullivan, S. D. (2023). *Outcomes-based contracting experience: Research findings from U.S. and European stakeholders. Journal of Health Economics and Outcomes Research, 11*(2), 153–164. https://doi.org/10.36469/jheor.2023.153

Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10398222/

[32] Chernew, M. E., et al. (2023). *Outcomes-based contracting with value-based insurance design: Opportunities to drive more health per pharmacy dollar*. University of Michigan V-BID Center. Retrieved from https://vbidcenter.org/outcomes-based-contracting/

[33] ForHealth Consulting (University of Massachusetts Medical School). (2024). *Exploring the trend of value-based contracts in the United States.* Retrieved from https://forhealthconsulting.umassmed.edu/exploring-the-trend-of-value-based-contracts-in-the-united-states/

[34] Office of Health Economics (OHE). (2023). *Indication-based pricing.* Retrieved from https://www.ohe.org/topic/indication-based-pricing/

[35] Lyfegen. (2024). *Indication-specific pricing to make inroads in the U.S.* Retrieved from https://www.lyfegen.com/post/indication-specific-pricing-to-make-inroads-in-the-u-s

[36] Institute for Clinical and Economic Review (ICER). (2016). *Indication-specific pricing of pharmaceuticals in the United States health care system. Policy Summit Report.* Retrieved from https://icer.org/wp-content/uploads/2021/01/Final-Report-2015-ICER-Policy-Summit-on-Indication-specific-Pricing-March-2016_revised-icons-002.pdf

[37] IQVIA Institute. (2023). *Success multiplied: Launch excellence for multi-indication assets.* Retrieved from https://www.iqvia.com/-/media/iqvia/pdfs/library/white-papers/iqvia-launch-excellence-for-multi-indication-assets-02-23-forweb.pdf

[38] Umbrex. (2024). *Global drug launch strategy and sequencing.* Retrieved from https://umbrex.com/resources/industry-analyses/how-to-analyze-a-pharmaceutical-company/global-drug-launch-strategy-and-sequencing/

[39] Oregon Health Authority. (2024). *Oregon value-based payment compact: Final report 2024.* Retrieved from https://www.oregon.gov/oha/HPA/dsi-tc/Documents/VBP-Compact-2024-Final-Report.pdf

[40] Organisation for Economic Co-operation and Development (OECD). (2019). *Pharmaceutical innovation and access to medicines.* OECD Publishing. https://doi.org/10.1787/9789264307391-en Retrieved from https://www.oecd.org/en/publications/pharmaceutical-innovation-and-access-to-medicines_9789264307391-en.html

[41] Khandelwal, N., & Chambers, J. D. (2023). *The current status of outcomes-based contracting for manufacturers and payers: An AMCP membership survey. Journal of Managed Care & Specialty Pharmacy, 29*(5), 463–472. https://doi.org/10.18553/jmcp.2023.29.5.463 Retrieved from https://pmc.ncbi.nlm.nih.gov/articles/PMC10397909/

[42] Bell, R., Et al. (2023). *Delivering the triple win: A value-based approach to pricing. Office of Health Economics.* Retrieved from https://www.ohe.org/wp-content/uploads/2023/04/Bell-et-al.-2023-A-Value-based-Approach-to-Pricing.pdf