AIMEDALLIANCE

EXECUTIVE SUMMARY

INTERNATIONAL REFERENCE PRICING:
Lessons Learned from Abroad

BACKGROUND

For decades, rising costs of health care, health insurance, and prescription drugs have been a major concern for American consumers, caregivers, providers, and employers. To address these challenges, policymakers are exploring international reference pricing (IRP) models, such as the proposed "Most Favored Nations" (MFN) policy, and more formalized models such as the GENEROUS (GENErating cost Reductions fOr U.S. Medicaid) Model, GUARD (the Guarding U.S. Medicare Against Rising Drug Costs) Model, and the GLOBE (Global Benchmark for Efficient Drug Pricing) Model. While the details of each approach differ, these models all propose to align United States (U.S.) prescription drug costs with those paid by comparable countries within the Organization for Economic Co-operation and Development (OECD).

To build consensus on the potential impact of an IRP pricing model in the U.S., Aimed Alliance convened a consensus meeting with international patient group stakeholders and advocates representing patients, providers, and caregivers from Europe, Oceania, and North America, to explore patient-centered alternatives and identify best practices in international value assessments. Participants offered valuable insights that highlighted key concerns of international patients, shared best practices, and provided actionable recommendations to addressing prescription drug affordability.

This executive summary outlines the white paper's key findings on the opportunities and challenges of implementing an IRP model in the U.S.

KEY FINDINGS

U.S. health care costs and prescription drug spending continue to outpace inflation and place substantial financial burdens on patients. Federal reforms such as the Affordable Care Act (ACA) and the Inflation Reduction Act (IRA) have provided some relief, but affordability gaps persist, with many Americans reporting medication nonadherence due to out-of-pocket costs.

To address these challenges, the current Trump Administration is considering international reference pricing to align U.S. drug prices with those in OECD countries; however, substantial differences in health systems, value assessment methods, and legal frameworks present major challenges to direct adoption and could harm U.S. consumer access to treatments and care.

Differences in Health Systems

Participants noted that key differences in private and public health care systems and value assessment frameworks distinguish the U.S. health care system from OECD countries. Under single-payer, government-funded systems, consumers have more experience with slower access to treatments and providers, with many participants citing international challenges such as long wait times for providers and specialists. As a result, patients who could afford care outside their home country often sought it elsewhere.

In contrast, participants noted that U.S. patients receive both care and treatments faster. For example, a RAND study found that of 287 new drugs introduced in 2022, U.S. consumers had access to 74%, compared to 52% in Germany, the next highest. Moreover, more than half of new drugs were first launched in the U.S., while major OECD countries like Australia, Canada, France, Germany, Italy, Japan, and the United Kingdom often experienced delays of nearly 12 months. The study recognized that the absence of reference pricing in the U.S. may contribute to its role as the primary launch market. However, participants also acknowledged that this advantage can come with higher costs for U.S. patients.

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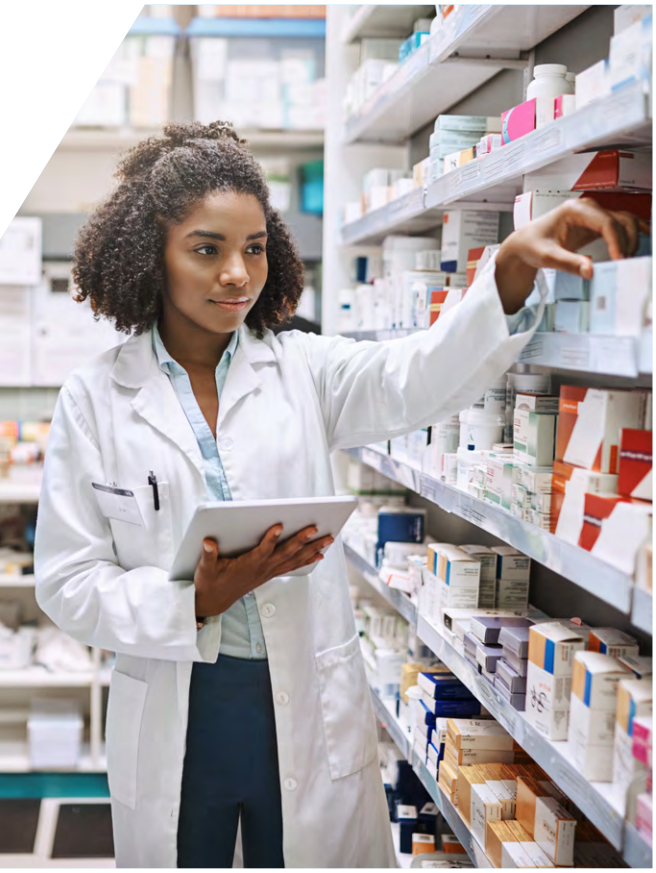
Ultimately, participants recognized that neither system is perfect for patients, and each government must pursue reforms that reflect its own unique challenges – whether affordability, access to specialists, or timely treatment – countries, including the United States, are best served by solutions tailored to the specific needs of their patients, caregivers, and providers.



Value Assessment Frameworks – QALYs and Patient Values

Participant discussions focused on variations in value assessment frameworks and the entities conducting these assessments. As the only country with a multi-payer system (Medicaid, Medicare, commercial insurance, and employer-sponsored health plans), U.S. pricing varies substantially compared to OECD nations. Most OECD countries reviewed in this paper rely on health technology assessments (HTAs), typically incorporating quality-adjusted life-years (QALYs) into their frameworks. However, U.S. federal law prohibits the direct or indirect use of QALY data in programs managed under the Social Security Act. As such, participants noted there could be potential challenges in adopting an OECD country's pricing analysis that relies on QALY evaluations.

Participants also recognized the importance of HTAs in drug pricing systems, but emphasized that their effectiveness depends on the quality of the input they seek, consider, and incorporate. Participants noted that across international value assessment frameworks, patients consistently value access to novel treatments, yet regulatory or administrative agencies often fail to adequately consider and reconcile patient feedback with reimbursement and coverage decisions. Additional challenges included limited transparency and inconsistent patient engagement policies and requirements, resulting in value assessment decisions that do not necessarily reflect the patient-perceived value of these treatments. Thus, participants warned that adopting foreign comparators imports their underlying assumptions, potentially perpetuating any misinterpretations and discriminatory metrics used in the original assessments in the U.S.

While a majority of countries conduct individual value assessments, a smaller number rely on international reference pricing. Of the country practices reviewed, participants noted that there was a lack of transparency in how countries selected their reference price comparators. In addition, they raised concerns that reference pricing often prioritizes achieving the lowest price rather than the fairest price. For example, the Netherlands previously used Germany as a reference country, but later substituted it with Norway after deeming Germany's prices too high. This is an important distinction. A fair price reflects the value a drug provides to patients, caregivers, and providers, what the market can reasonably pay, and the investment required to develop it. In contrast, the lowest possible price focuses on what a system can pay, often placing less consideration on the

infrastructure, research, and innovation necessary to bring the drug to market. Participants noted that for U.S. policymakers, this issue is critical as the need for an IRP model is based on the principle that Americans bear an unfair share of these global costs.

Thus, when evaluating IRP adoption, policymakers should consider which policies and approaches will ensure *fair* global pricing, which countries seek to balance affordability with sustainability, and which focus solely on securing the lowest costs regardless of global and patient consequences.

Rare Disease Communities

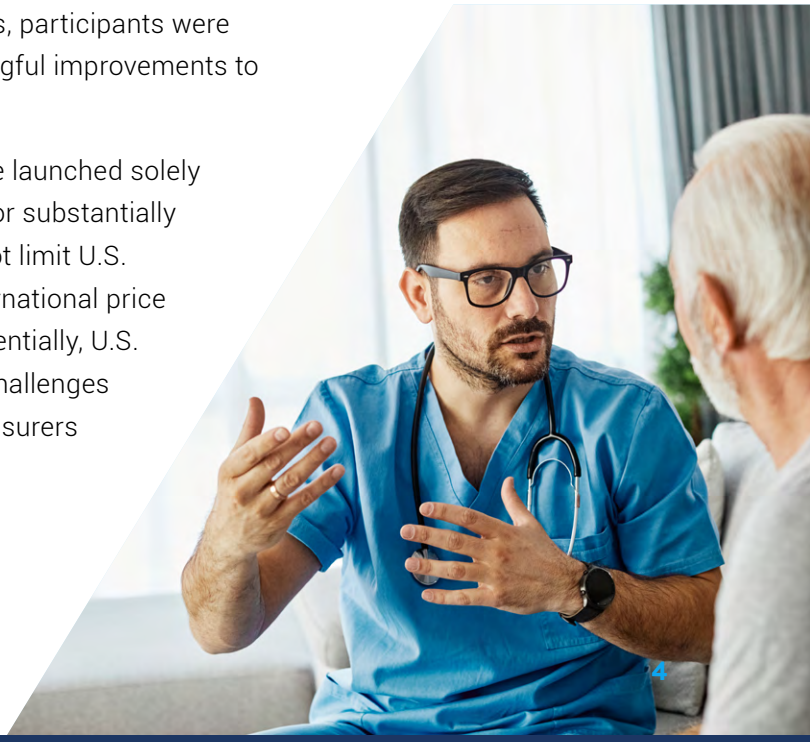
Although definitions of rare disease thresholds vary across countries, participants universally acknowledged the global challenges facing rare disease communities in research and development. Incentivizing innovation for rare disease treatments remains difficult, and reimbursement decisions are often complicated by small patient populations and limited clinical trial data. Fortunately, a 2019 survey found that private insurers in the U.S. cover these treatments in 99% of cases. This access contrasts many OECD countries, which have substantially less coverage. For example, in Spain, fewer than 67% of orphan drugs approved for use in Europe are covered and reimbursed. Thus, rare disease patients often have less access to these treatments in OECD countries. In response, many OECD countries have begun revising value assessment frameworks to exclude rare disease treatments, recognizing that these therapies rarely meet QALY-based thresholds.

Participant discussions largely focused on broad best practices and pricing assessments rather than rare disease-specific frameworks, which are becoming increasingly distinct from general prescription drug evaluations. As such, participants recognized the need for further research to identify best practices for assessing the value of rare disease treatments.

Impact on Innovation & Patients

IRP models could have far-reaching global consequences, including slowing research and development, delaying or limiting launches in comparative countries, increasing prices abroad, and even prompting therapy withdrawals. Participants emphasized that these implications could negatively affect global patient communities. Moreover, without regulatory language mandating savings from IRP models be passed down to consumers, participants were concerned if an implemented IRP would provide meaningful improvements to affordability for U.S. consumers.

Participants also had concerns that new drugs would be launched solely in the U.S. and novel treatments would be inaccessible or substantially delayed globally. While delayed launches abroad may not limit U.S. access, it will impact affordability, because without international price comparators, any IRP model would be inapplicable. Essentially, U.S. consumers would stay stuck in the same affordability challenges while key drivers of health care costs, such PBMs and insurers would continue to operate unchecked.



MOVING FORWARD: U.S.-Based Solutions

Given the unique structure of the U.S. health care system, reforms should prioritize measures that deliver meaningful benefits to U.S. consumers, payers, and providers. U.S. prescription drug affordability is a challenge driven by factors unique to the U.S. health care system, requiring solutions tailored specifically to addressing those root causes. Participants identified the following reforms that would be more impactful than IRP models:

Investigate International PBM Aggregators:

The U.S. Trade Representative (USTR) should examine whether PBM aggregators are operating internationally to circumvent U.S. laws requiring prescription drug rebates to be passed to payers and employers. USTR should investigate these practices and take appropriate actions to ensure all savings reach consumers.

Consider Medicaid Carve-Outs for Prescription Drugs:

States should explore carving out Medicaid prescription drug benefits from managed care contracts to increase savings and improve affordability. Eight states have already implemented carve-outs, generating substantial savings. For example, New York saved an estimated $400 million in 2024 by independently managing its formulary and rebates.

Strengthen Pharmacy Benefit Manager (PBM) Oversight:

PBMs are third-party middlemen that play a major role in the U.S. drug pricing system yet remain largely unregulated by state or federal authorities. Reform should include increasing transparency on drug costs, prices, markups, and discounts; banning spread pricing; implementing delinking practices; and imposing fiduciary duty obligations.

Prioritize Systematic Reform over Individual Manufacturer Agreements:

While individual arrangements between the White House and pharmaceutical companies demonstrate good-faith efforts, long-term affordability improvements require reform addressing institutional challenges such as PBM practices, insurer practices, and other U.S. specific third-party cost drivers.

Address Consumer Out-of-Pocket Costs:

Reforms that directly lower out-of-pocket costs will have the greatest impact on consumers. Options include annual out-of-pocket caps similar to those in the IRA or banning copay accumulator programs.

MOVING FORWARD: Patient-Centered International Reference Pricing

If the Trump Administration proceeds with international reference pricing, the following recommendations should be implemented to ensure patients, caregivers, and providers can meaningfully participate in and shape the process.

Use IRP as a Benchmark, Not a Baseline:

Any use of an international reference price should supplement, not replace, an independent U.S. pricing assessment that reflects federal law and anti-discrimination protections, U.S. patient values, and the U.S. patient populations.

Prohibit the Use of Discriminatory QALYs:

Federal law bans the direct and indirect use of QALY data in federal programs governed by the Social Security Act. Policymakers should ensure this prohibition is upheld in any international reference pricing program and applies to all federal programs, given the discriminatory nature of these assessments.

Ensure Patient Engagement from the Earliest Stages:

Developing a pricing system that accurately reflects patient needs requires involving patients, providers and caregivers from the beginning. Early engagement substantially improves the process and leads to more equitable and accurate outcomes. As one participant noted, "patients should be placed at the center of the healthcare system and that this participation should be structural, early and binding, not just consultative." Engaging patients from the outset prevents the need to later overhaul systems to better reflect U.S. patient values, views, and communities.

Provide Opportunities for Patient Feedback and Reconcile Feedback in Decisions:

A persistent global challenge is the lack of transparency around how decision-makers use patient, caregiver, and provider feedback. Any IRP programs should allow patients to comment on drug value and require decision-makers to explain how input and insight informed final pricing decisions.

Protect Rare Disorders:

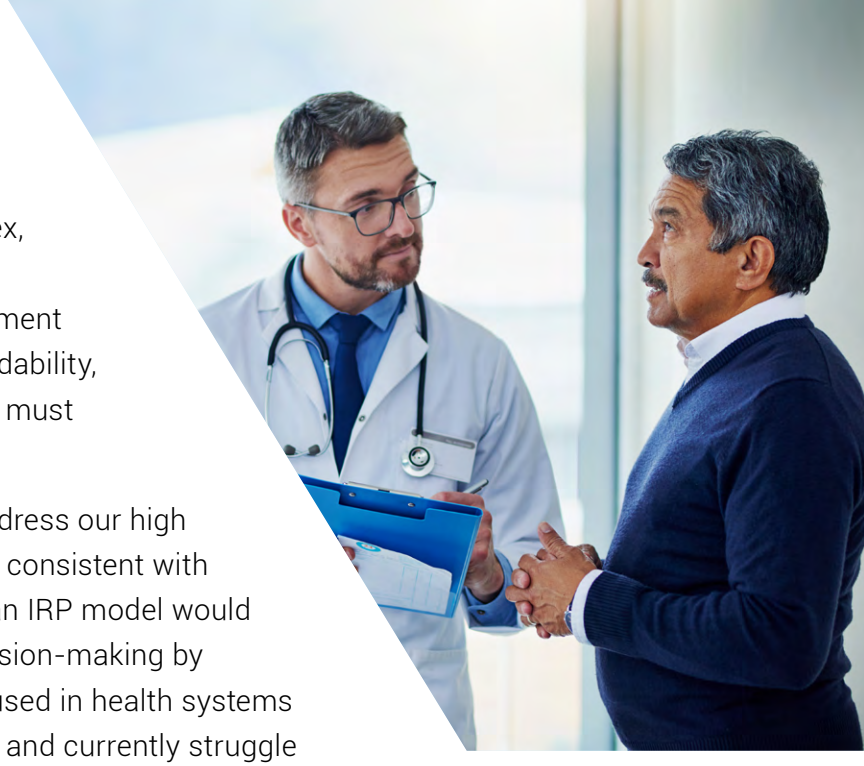
Treatments for rare diseases, disorders, and orphan drugs should be carved out from IRP models. Rare disease therapies often fail QALY thresholds due to the small patient population sizes. Participants noted that, given these distinct challenges and needs of the rare disease community, additional research is needed to establish value-assessment best practices for these communities.

Protect Innovation:

The U.S. is a global leader in biotechnology and pharmaceutical innovation, with many novel treatments launching in the U.S. before other countries. This leadership is critical for U.S. patients who depend on timely access to cutting-edge treatments. An IRP program could unintentionally hinder research, development, and access. Safeguards to protect this infrastructure could include:

- **Create a small biotech exemption:** Similar to the IRA, an exemption could reiterate Congress's concerns about small biotechnology companies and the need to safeguard these stakeholders to ensure continued investing in research and development for new treatments.
- **Delay the application of international reference pricing:** Under the IRA, Medicare price negotiations are delayed for seven years after FDA approval for small-molecule drugs and 11 years for large molecule drugs. However, health policy experts have raised concerns that these differing timelines may disadvantage investment in small-molecule drugs. Congress is therefore considering legislation to establish a single, uniform negotiations timeline and legislation that clarifies how niche technologies, like genetically targeted therapies, fall within these timeframes. If policymakers adopt an IRP program, they should similarly consider implementing a uniform negotiation timeline across all therapeutics.
    - Without such safeguards, manufacturers may choose to launch certain products exclusively in the U.S. and delay introductions in other countries to avoid triggering reference pricing benchmarks.
- **Create pricing control exceptions:** IRP operates with the understanding that one pharmaceutical company controls global pricing for a product. In practice, many smaller companies license or sell the rights to manufacture and market their products to international companies. Under these circumstances, it would be difficult for the original company to be held accountable for IRPs it cannot influence. Policymakers should therefore consider whether and how such companies should be subject to an IRP requirements and whether targeted exceptions are appropriate.

CONCLUSION



The U.S. health care system is uniquely complex, diverse, and centered on patient choice, rapid access to innovation, and a wide range of treatment options. Any effort to improve health care affordability, and prescription drug affordability in particular, must reflect these realities.

Although the U.S. urgently needs reforms to address our high prescription drug costs, these reforms must be consistent with how the U.S. values and federal law. Adopting an IRP model would move the U.S. away from patient-centered decision-making by importing foreign pricing frameworks that are used in health systems that operate inherently differently from the U.S. and currently struggle to meaningfully incorporate patient insights in reimbursement decisions, resulting in diminished access and coverage for patients.

Patients, providers, and caregivers need practical and meaningful reform that addresses the barriers within our unique system that are driving costs, such as PBM practices, insurer benefit design, and lack of transparency. In conclusion, U.S. policymakers should pursue solutions that improve affordability without sacrificing the principles that matter most to its patients: choice, timely access, and meaningful involvement in decisions that fundamentally shape their lives.

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