Amgen's Response to Icer's Proposed Value Framework

A Technical Analysis of Healthcare Value Assessment, Orphan Drug Policy, and Patient-Centered Access

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EXECUTIVE SUMMARY

Healthcare value assessment frameworks have become increasingly important in determining how pharmaceutical therapies are evaluated, priced, reimbursed, and accessed within modern healthcare systems. As healthcare costs continue to rise and pharmaceutical innovation becomes more advanced, healthcare organizations and policymakers are increasingly relying on economic evaluation models to assess whether therapies provide sufficient clinical and societal value relative to their cost. One of the most influential organizations in this field is the Institute for Clinical and Economic Review (ICER), which develops evidence-based cost-effectiveness assessments that influence healthcare coverage decisions, reimbursement strategies, and pricing discussions across the pharmaceutical industry.

This paper examines Amgen's response to ICER's proposed adaptation of the ICER Value Framework, particularly regarding its application to orphan and ultra-orphan disease therapies. According to Amgen, the proposed framework may unintentionally restrict patient access to innovative treatments by applying valuation methods that may not fully reflect the scientific, clinical, and societal complexities associated with rare disease therapies. The company argues that orphan drug development involves unique challenges, including limited patient populations, high research and development costs, small clinical trial datasets, and significant uncertainty regarding long-term therapeutic outcomes. As a result, traditional cost-effectiveness models may inadequately measure the full value of these therapies.

The article explores several key areas, including ICER's healthcare value assessment methodology, rare disease and orphan drug policy, patient-centered healthcare evaluation, healthcare economics, and ethical concerns associated with value-based healthcare systems. It also examines how cost-effectiveness models such as Quality-Adjusted Life Years (QALYs) are used to assess therapeutic value and why these methodologies remain controversial within the biotechnology and healthcare sectors.

Additionally, the paper discusses the importance of maintaining innovation incentives in biotechnology and pharmaceutical research. Amgen argues that overly restrictive reimbursement frameworks may discourage investment in rare disease research and reduce the development of future therapies for underserved patient populations. The paper further highlights the growing demand for more patient-centered healthcare models that incorporate caregiver burden, quality-of-life improvements, and broader societal impacts into healthcare decision-making.

Beyond Amgen's position, this paper also evaluates the broader implications of healthcare value assessment frameworks for pharmaceutical companies, healthcare providers, insurers, regulators, policymakers, and patients living with rare diseases. Ultimately, the discussion reflects the larger challenge facing modern healthcare systems: balancing healthcare affordability and economic sustainability while continuing to support medical innovation, equitable treatment access, and patient-centered care.

INTRODUCTION

Healthcare systems worldwide are increasingly focused on balancing clinical innovation, patient access, and cost sustainability. As pharmaceutical therapies become more advanced and personalized, healthcare organizations and policymakers have adopted value assessment frameworks to evaluate the clinical and economic impact of new medical treatments.

One of the most influential organizations in this area is the Institute for Clinical and Economic Review (ICER), an independent nonprofit organization that evaluates healthcare interventions using evidence-based cost-effectiveness models. ICER assessments are frequently referenced by payers, insurers, policymakers, and healthcare organizations when making pricing and reimbursement decisions.

In response to ICER's proposed adaptation of its value assessment framework for orphan and ultra-orphan diseases, Amgen issued a formal statement expressing concerns about the potential consequences of the revised methodology. According to Amgen, the proposed framework could negatively affect patients with rare diseases by creating restrictive valuation standards that may reduce incentives for orphan drug development and limit access to innovative therapies.

Amgen's response highlights broader debates within the healthcare industry regarding how value should be defined, measured, and applied in pharmaceutical decision-making.

UNDERSTANDING ICER AND THE VALUE ASSESSMENT FRAMEWORK

The Institute for Clinical and Economic Review (ICER) develops evidence-based assessments designed to evaluate the clinical effectiveness and economic value of healthcare interventions.

The organization uses cost-effectiveness analysis (CEA) methodologies, including metrics such as Quality-Adjusted Life Years (QALYs) and Equal Value of Life Years Gained (evLYG), to estimate the value of therapies relative to their costs.

ICER's framework aims to support:

- Fair pricing
- Sustainable healthcare spending
- Evidence-based reimbursement
- Improved patient outcomes

According to ICER, the framework is intended to create a transparent and systematic process for evaluating healthcare value across multiple therapeutic categories.

However, pharmaceutical manufacturers and patient advocacy organizations have raised concerns regarding several aspects of the framework, including:

- Reliance on population-level cost-effectiveness metrics
- Limited inclusion of patient perspectives
- Challenges in measuring long-term therapeutic value
- Difficulties evaluating rare disease therapies
- Potential undervaluation of innovation

These concerns become particularly significant in the context of orphan and ultra-orphan diseases, where small patient populations create unique clinical and economic challenges.

AMGEN'S POSITION ON ICER'S PROPOSED ADAPTATION

Amgen's response emphasizes that ICER's proposed adaptation for orphan disease assessment could conflict with the intent of the U.S. Orphan Drug Act (ODA), which was established to encourage development of therapies for rare diseases affecting fewer than 200,000 individuals in the United States.

One of Amgen's primary concerns involves ICER's proposed distinction between "ultra-orphan" and "non-ultra-orphan" diseases. ICER suggested using a threshold of approximately 10,000 patients to define ultra-rare conditions, while other orphan diseases would be evaluated under more conventional value assessment approaches.

Amgen argues that this distinction creates several problems:

Redefinition of Rare Disease Criteria

Amgen states that ICER's proposed thresholds effectively redefine orphan disease classifications beyond the definitions established by federal legislation. The company argues that this approach may disadvantage patients with rare diseases that exceed ICER's proposed ultra-rare threshold but still qualify as orphan diseases under U.S. law.

Reduced Incentives for Innovation

The company also argues that restrictive valuation models may discourage investment in rare disease research and development. Orphan drug development often involves:

- High research costs
- Small patient populations
- Limited commercial markets
- Complex clinical trial requirements
- Significant scientific uncertainty

Amgen maintains that reducing reimbursement potential could weaken incentives for biotechnology innovation in rare disease therapeutics.

Insufficient Patient Representation

Another major concern involves patient-centered evaluation. Amgen argues that rare disease patients and caregivers should play a larger role in determining therapeutic value and treatment priorities.

THE IMPORTANCE OF ORPHAN DRUG POLICY

The Orphan Drug Act of 1983 was enacted to stimulate research and development for rare diseases that historically received limited pharmaceutical investment due to small market sizes.

The legislation provides incentives including:

- Market exclusivity
- Tax credits
- Research grants
- Regulatory assistance

These protections were designed to address market failures associated with rare disease drug development.

According to Amgen, ICER's proposed framework could undermine some of these incentives by applying traditional cost-effectiveness methodologies to therapies developed under fundamentally different economic and scientific conditions.

Rare disease therapies often involve:

- Limited patient populations
- Smaller clinical trial datasets
- Higher manufacturing costs
- Increased uncertainty in long-term outcomes
- Specialized treatment infrastructure

Traditional cost-effectiveness models may not fully capture the broader societal and patient benefits associated with these therapies.

PATIENT-CENTERED HEALTHCARE AND VALUE MEASUREMENT

A central theme in Amgen's response is the importance of patient-centered healthcare evaluation.

Traditional health economic models often emphasize population-level efficiency metrics, but critics argue that these models may fail to capture:

- Caregiver burden
- Quality-of-life improvements
- Disease severity
- Societal productivity gains
- Psychological and emotional benefits
- Long-term functional outcomes

Recent updates to ICER's framework have attempted to incorporate broader societal considerations, including patient productivity and caregiver impact.

However, Amgen argues that these modifications remain insufficient for accurately assessing rare disease therapies.

The company recommends:

- Greater patient participation in assessments
- Inclusion of broader societal value elements
- Increased use of real-world evidence
- More flexible evaluation methodologies for rare diseases

These recommendations reflect broader industry efforts to shift healthcare evaluation toward more holistic and patient-centered frameworks.

ECONOMIC AND REGULATORY CONSIDERATIONS

Healthcare value assessment frameworks increasingly influence:

- Insurance reimbursement decisions
- Formulary placement
- Coverage restrictions
- Government pricing negotiations
- Investment strategies within biotechnology

As healthcare spending continues to rise, policymakers and insurers seek mechanisms to improve cost efficiency while maintaining access to innovative therapies.

However, pharmaceutical companies argue that overly restrictive pricing models may reduce incentives for future innovation.

This debate has intensified following implementation of the Inflation Reduction Act (IRA), which introduced Medicare drug price negotiation mechanisms in the United States. Several industry analysts have suggested that ICER's framework could indirectly influence future pricing negotiations and reimbursement strategies.

The challenge for policymakers involves balancing:

- Affordable healthcare access
- Sustainable healthcare spending
- Continued pharmaceutical innovation
- Patient-centered treatment availability

CHALLENGES IN COST-EFFECTIVENESS MODELS

Cost-effectiveness analysis remains one of the most debated aspects of healthcare economics.

Metrics such as QALYs are designed to estimate the value of therapies by measuring both survival and quality-of-life improvements. However, critics argue that these models may unintentionally disadvantage:

- Disabled populations
- Elderly patients
- Rare disease patients
- Individuals with chronic illnesses

Several organizations have expressed concerns regarding whether QALY-based methodologies fully reflect patient experiences and ethical considerations.

Additional limitations include:

Data Uncertainty

Rare disease therapies often rely on limited clinical datasets due to small patient populations.

Long-Term Benefit Measurement

Some therapies provide benefits over decades, making long-term valuation difficult.

Societal Value Complexity

Economic models may struggle to quantify productivity gains, caregiver relief, and broader social outcomes.

Equity Considerations

Strict population-level cost-effectiveness thresholds may conflict with ethical principles surrounding access to care for vulnerable populations.

Amgen argues that healthcare value assessment frameworks should remain flexible enough to accommodate these complexities.

STRATEGIC INDUSTRY IMPLICATIONS

The debate surrounding ICER's framework reflects larger transformations occurring across the pharmaceutical and healthcare industries.

Biotechnology companies increasingly operate within environments shaped by:

- Value-based healthcare systems
- Regulatory pricing pressure
- Expanded health technology assessment (HTA)
- Outcome-based reimbursement models
- Precision medicine strategies

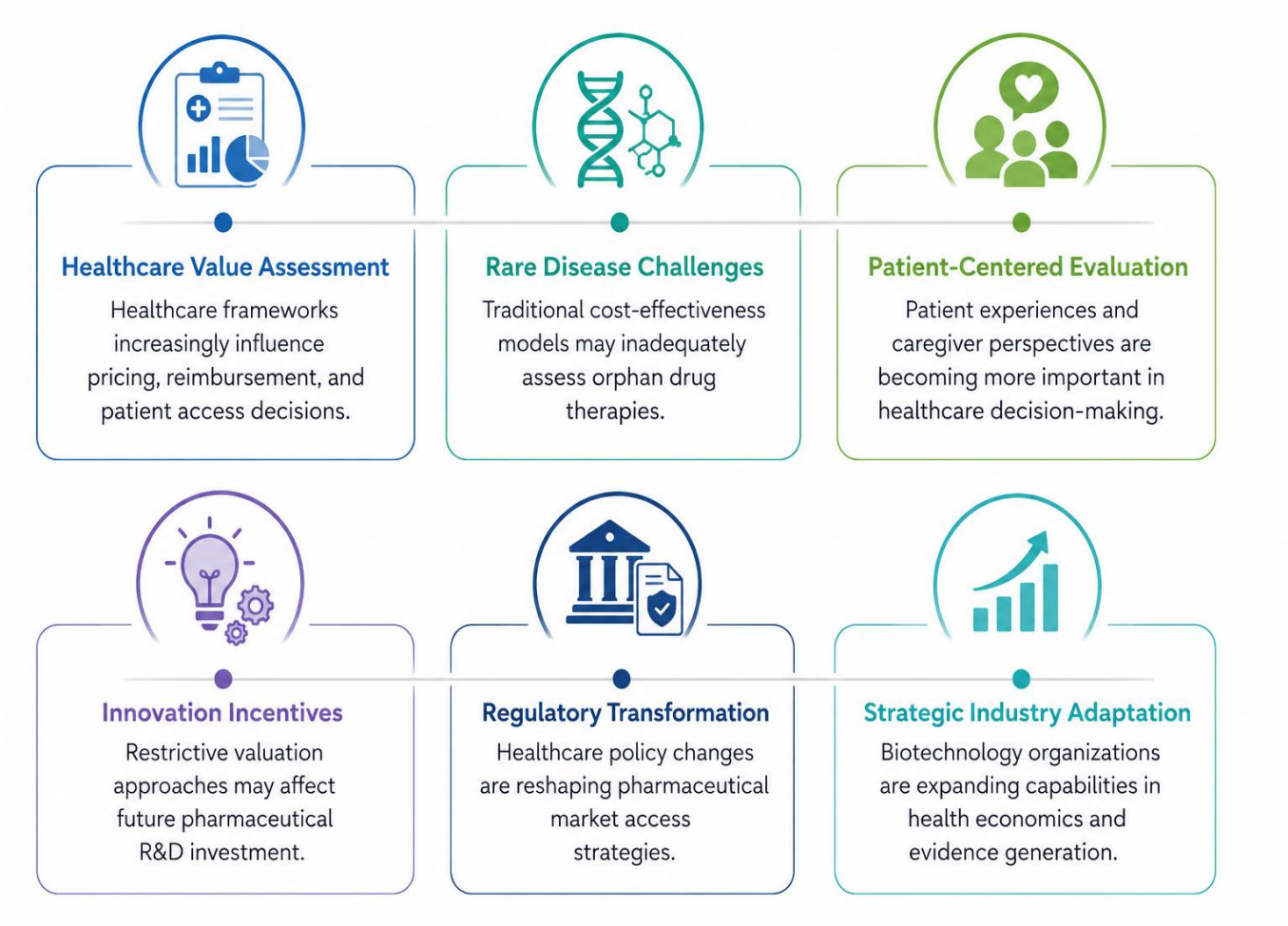
Organizations must now demonstrate not only clinical efficacy but also economic and societal value.

As a result, pharmaceutical companies are investing more heavily in:

- Real-world evidence generation
- Patient-reported outcomes
- Health economics and outcomes research (HEOR)
- Data analytics and AI-assisted healthcare modeling

Amgen's response illustrates how manufacturers are actively engaging in policy discussions to influence future healthcare evaluation standards.

KEY TECHNICAL INSIGHTS



This visual presentation highlights the key issues surrounding healthcare value assessment and pharmaceutical innovation. It emphasizes the challenges of evaluating rare disease therapies, the growing importance of patient-centered healthcare, and the impact of regulatory and economic frameworks on biotechnology research and market access.

CONCLUSION

Amgen's response to ICER's proposed adaptation of the value assessment framework highlights the growing tension between healthcare affordability, evidence-based evaluation, and continued pharmaceutical innovation.

While healthcare stakeholders increasingly seek sustainable cost-management strategies, pharmaceutical companies and patient advocacy organizations argue that value assessment frameworks must remain flexible enough to account for the complexities associated with rare disease therapies and biologic innovation.

The debate also reflects broader shifts toward patient-centered healthcare evaluation, expanded use of real-world evidence, and more holistic approaches to measuring therapeutic value.

As healthcare systems continue evolving, organizations such as ICER, pharmaceutical manufacturers, regulators, and patient groups will likely play increasingly important roles in shaping future standards for healthcare value assessment.

The challenge moving forward will be developing methodologies that balance:

- Scientific rigor
- Economic sustainability
- Ethical fairness
- Innovation incentives
- Patient access

Ultimately, the future of healthcare value assessment will depend on whether stakeholders can create systems that support both medical innovation and equitable access to life-changing therapies.

REFERENCES

1. Amgen. “Amgen Response to ICER’s Proposed Adaptation of the ICER Value Framework.”

2. Amgen. “Amgen Comments on ICER’s Proposed Changes to the 2020 Value Assessment Framework.”

3. Institute for Clinical and Economic Review (ICER). “Value Assessment Framework.”

4. Syneos Health. “What Can We Learn From ICER’s New Value Framework?”

5. Managed Healthcare Executive. “ICER Updates its Assessment Framework to Include New Measures of Value.”

6. Avalere Health Advisory. “ICER Opens Brief Comment Period on Proposed Framework Updates.”

7. Marketscreener. “Amgen Comments on ICER’s Proposed Methods Adaptations.”