



PATIENT ACCESS & AFFORDABILITY PROJECT

Empowering Patient Perspective.

HEALTH TECHNOLOGY ASSESSMENT BEST PRACTICES FOR FOR RARE DISEASE DRUGS

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Preamble

Health Technology Assessment (HTA) practices are currently intended to express projected costs and benefits of new medical technologies and therapeutics that treat diseases. They are used by payers to inform policy, reimbursement and patient access decisions. To assess the value of a new technology or therapy, HTA bodies currently use generalized quality of life models, rather than fundamental scientific measures that are disease and/or patient specific and supported by empirical evidence. HTA assessments seek to conduct a cost-benefit analysis, but there are important costs that they do not weigh in their modeling. HTA bodies do not adequately assess indirect medical costs to families and society like caregiving expenses, home modifications, need for a personal aid, among other expenses to treat the disease not paid for by a health insurer. Non-health related economic outcomes, such as productivity regained or the ability to return to school, or a caregiver's ability to return to work are also not considered.

By not adapting to specific diseases, HTA assessments often fail to illustrate the comprehensive clinical, economic and social value that patients, families, caregivers and society at-large experience from a new treatment. This is most evident in HTA evaluations of rare disease therapies, where collecting data and generating evidence through traditional clinical trial designs is complicated, if not impossible, due to small patient groups, diseases that are heterogeneous, and few existing treatment options.

Rare disease communities experience unique challenges that affect patients and their families. Among the challenges are struggles of being diagnosed, receiving optimal care, and accessing treatment. More than 90 percent of rare diseases do not have treatment that is approved by the Food and Drug Administration. This impacts a large number of patients worldwide. In the U.S. alone, 30 million Americans are living with a rare disease and half are children, according to the [National Organization for Rare Disorders](#).

Generating evidence through traditional randomized clinical trials (RCT) is often impractical or unethical for rare disease drugs. For rare disease therapies a traditional RCT can be hampered

simply because of limited patient enrollment in a clinical trial due to small patient populations. Other reasons could be a limited understanding of a disease's natural history or that enrolling patients in a sham placebo arm is unethical because the outcome is rapid degeneration and/or certain death.

It is critical that regulators and HTA bodies adopt a new framework for rare disease therapies that is scientifically robust, flexible and patient-centric. The aim should be a system that provides rare disease patients with a more rapid process for regulatory approval and market access to cutting-edge medical innovation, while continuing research program that uses disease-specific measures to collect credible, empirical and replicable evidence of benefit and value throughout the life cycle of a treatment.

This paper outlines a new approach to HTA and establishes best practices to ensure rare disease treatments are valued appropriately, patients have access to FDA-approved therapies, and manufactures are responsible for conducting ongoing research and evidence generation.

Patients Need a Transparent, Collaborative, Adaptive and Equitable Process.

- Regulators, HTA bodies and payers should collaborate throughout the development and lifecycle of a therapy with patients, caregivers, clinicians and manufactures.
- HTA organizations should state in their final report how patient experience and related data - including information from product sponsor or a patient advocacy organization - were quantitatively applied in their assessment of an FDA-approved treatment.
- Disease specific specialists that understand what constitutes patient value should be active participants in any drug assessment.
- HTA value assessment frameworks should aim to improve health equity and consider the value new medical technologies may provide in terms of reducing health disparities among racial and ethnic minority groups, and people with disabilities.

Science-Based Value Claims Should be the Basis for Pricing and Access Decisions

- HTA value assessment frameworks should abandon the quality adjusted life year (QALY) and similar discriminatory tools when determining economic value or to set a value-based price.
- The foundation of an HTA framework should be the evaluation of science-based value claims, proposed in both pivotal clinical trials and for ongoing real world data collection, that are used as the basis for coverage and access decisions.

- Value frameworks must be adaptable to the disease and based on what matters to patients and caregivers.
- Manufacturers should begin establishing value claims for rare disease treatments prior to FDA-approval with a detailed assessment of the target patient population, along with the unmet medical and evidence needs.
- All value claims, including Patient Reported Outcome measures, should be scientifically valid - expressed in ratio or interval form- and disease-specific to collect credible, empirical and replicable evidence of benefit and value.

Encourage Evidence Generation.

- Given the limited data at approval, manufacturers, HTA bodies, payers and policymakers should focus on long term research programs that generate a scientifically robust evidence base overtime with manufactures commitment to ongoing value claim assessments.
- Post-approval commitments, like confirmatory trials, should be a simple, collaborative and realistic effort with manufacturers and stakeholders to increase evidence generation.
- Data systems and patient registries should be developed so that they can capture patient-reported outcomes reflecting broader patient and family effects of treatment.
- It is the role of the payer to weigh the totality evidence of value claims for a target patient population and to factor these claims into pricing and access recommendations.
- Payers should uphold the FDA's authority in determining safety and efficacy of the population included in the drug's FDA-approved indication statement.
- The price of a new rare disease treatment should be mutually agreed upon between the payer and manufacturer - and any cost sharing with patients should be minimal.
- Manufacturers should be open to disease area and therapeutic class reviews as evidence builds over its patent life or life cycle of a therapy.

The Best Practices were developed by Patients Rising with support from the Patient Access & Affordability Project's Rare Disease Health Technology Assessment Working Group. The mission of Patients Rising's Patient Access & Affordability Project is to provide patient-powered pathways to help legislators, regulators, payers, and employers better understand the patient experience as they make critical coverage decisions for patients with rare and chronic diseases.