PATIENTS RISING NOW

February 1, 2023

The Honorable Brett Guthrie
Chairman
Health Subcommittee
Washington, DC 20515

The Honorable Anna Eshoo
Ranking Member
Health Subcommittee
Washington, DC 20515

The Honorable Cathy McMorris Rodgers
Chairwoman
Energy and Commerce Committee
Washington, DC 20515

The Honorable Frank Pallone
Ranking Member
Energy and Commerce Committee
Washington, DC 20515

Dear Chairman Guthrie, Ranking Member Eshoo, Chairwoman Rodgers, and Ranking Member Pallone,

On behalf of Patients Rising Now, thank you for holding a legislative hearing focused on the *Protecting Health Care for All Patients Act*. Patients Rising Now is a national nonprofit patient advocacy organization driving efforts at the federal, state, and local level to ensure all people in the United States have **access** to high-value healthcare services and treatments with **transparent** and **affordable** pricing. Since our founding eight years ago, Patients Rising has provided people with chronic, rare, and life-threatening illnesses the information and services to best access affordable care.

We unequivocally support the full ban of Quality Adjusted Life Years (QALYs) in all government programs and Medicare valuations, which was, unfortunately, not included in the Inflation Reduction Act (IRA). QALY is not a household term, but its influence over patients' access to treatments and services looms large for the patient community.

The IRA sidesteps the problem of QALY use in value assessments by only prohibiting any measurement that "treats extending the life of an elderly, disabled, or terminally ill individual as of lower value than extending the life of an individual who is younger, non-disabled, or not terminally." This ambiguous wording is open to misinterpretation and fails to protect patients and their families from rationing and discrimination.

While care valuations may not directly discriminate against the elderly, disabled, or terminally ill, my fear is government programs will indirectly prejudice the most vulnerable. For example, Centers for Medicare and Medicaid Services currently restricts brain imaging in connection with diagnosing Alzheimer's disease to a single scan during a person's lifetime, which is contingent on that person's enrollment in a clinical trial. Many in underserved communities do

not have access to clinical trials. As a result, CMS has taken the advice of an FDA Advisory Committee and the Institute for Clinical and Economic Review (ICER) review in determining an entire class of current and future medications are not available to those who lack access to a clinical trial. This is just one example of the massive discrimination against seniors in underserved communities. Rationing decisions such as this set a dangerous precedent against future innovation.

In the absence of a national Health Technology Assessment (HTA) program, private entities like the Institute for Clinical and Economic Review (ICER) evaluate and determine the "value" of new therapies using QALY-based assessments. ICER and similar groups have a troubling pattern of ignoring the needs of patients living with rare diseases, resulting in decisions that would limit access and affordability for approved therapies.

Steve Pearson, ICER's President states that orphan drug spending places an "*undue burden … on others for the sake of a few*." Specifically, ICER asserts "*The opportunity cost of supporting the use of ultra-orphan drugs necessitates that patients with a more common disease, for which a cost-effective treatment is available, are denied treatment.*"

ICER's reports reduce every patient to a dollar amount, prioritizing the least costly patients rather than those with the greatest healthcare needs. And it isn't just academic economists like ICER that use the QALY. ICER is systematically leveraging its influence to use this model for care decisions for our veterans and Medicaid recipients.

QALY-based models view life as more valuable with a "perfect" body, in "perfect" health. If a patient does not meet HTA's desired characteristics, they will be disadvantaged in QALY-based equations. This is a payer-centric approach to healthcare finance, not a patient-centered model. Cancer patients and survivors, Rare Disease patients, the elderly, and disabled are disadvantaged by QALYs because lives are automatically discounted.

The QALY is a controversial metric that should be banned, but this issue of undervaluing patient's lives will require further action. There are more patient-centered and scientifically accurate ways to approach the value of treatments to the patient populations. We can start by including patients in developing new metrics. Their experiences will provide vital insights beyond the cold mathematical formula too often used in health care-design.

At the end of 2022, Patients Rising released a set of best practices for health technology assessment for rare disease treatments. This was developed over several months with our Patient Access and Affordability Project Working Group, and then endorsed by our Patient Delegates.

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It is a set of core principles, which we hope you will consider as you move forward in determining the most equitable ways to assess the value of treatments for patients. These best practices include:

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 a product sponsor or a patient advocacy organization - were quantitatively applied in the 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 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.

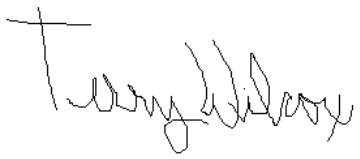
- Manufacturers should be open to disease area and therapeutic class reviews as evidence builds over its patent life or life cycle of a therapy.

In closing, I ask members of this committee to work across the aisle to find policy solutions that do not erect additional barriers to care for patients with rare and chronic diseases. We have a lot of work to do in creating *equitable and affordable healthcare for all patients in America*. This

starts with the design of the benefits that determine a patient's cost and access. Keep the QALY metric out of public benefits like Medicare.

Patients Rising Now strongly encourages the Energy and Commerce Committee to pass the *Protecting Health Care for All Patients Act* and ban the QALY from being used in any government program.

Sincerely,



Terry Wilcox

CEO and Founder