RESOLUTION ON ESTABLISHING A PATIENT-CENTERED FRAMEWORK FOR RARE DISEASE HEALTH TECHNOLOGY ASSESSMENT

WHEREAS, Health Technology Assessment (HTA) practices are currently intended to simulate cost effectiveness of new medical technologies and therapeutic and are used by payers to inform policy, reimbursement and patient access decisions, and

WHEREAS, HTA practices focus almost exclusively on health system costs and savings, and

WHEREAS, HTA bodies often use cost effectiveness assessments with assumption-based models and generalized quality of life estimates, like the Quality Adjusted Life Year (QALY), and

WHEREAS, HTA practices do not adequately assess indirect medical costs to families and society or non-health related economic outcomes, as was identified in The Economic Burden of Rare Disease study conducted by the Everylife Foundation, which showed that in calendar year 2019, the total economic burden of rare disease in the United States included $20B in transportation costs, $24B in necessary automobile modifications, $10B in necessary home modifications, $138B in lost productivity due to employees coming to work sick or disabled, $149B in employee absenteeism, $136B in lost productivity due to forced retirement, $38B in uncovered healthcare costs, $48B in outpatient Rx administration, $62B in outpatient costs, and

WHEREAS, the standard tool for patient reported outcomes within QALY calculations does not meet the FDA's standard for good measurement properties, and

WHEREAS, QALY advocates, like ICER and ISPOR acknowledge the QALY and cost model only capture part of the value and benefits of new therapies and interventions for rare disease treatments, and

WHEREAS, rare disease communities face unique challenges. At time of approval, many rare disease treatments have limited evidence, and

WHEREAS, generating evidence through traditional randomized clinical trials (RCT) is often impractical or unethical for rare disease drugs, and

WHEREAS, the rare disease community needs a more flexible approach that removes access barriers and establishes treatment value based on science, and

WHEREAS, HTA bodies currently use generalized quality of life models only capture part of the value and benefits of new therapies and interventions for rare disease treatments, now, therefore, be it

RESOLVED, the Patients Rising Delegates support the use of the Patient Access & Affordability Project's Best Practices for Rare Disease HTA by manufacturers, payers, regulators and policymakers to better serve the access needs of Rare Disease patients and to establish a new framework that is long-term, evidence-based and offers empirical reviews of a treatments value for patients, caregivers, and society-at-large.