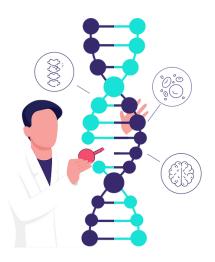


# Understanding the Value of Gene Therapies



Gene therapies have incredible potential to improve the lives of patients. As our healthcare system continues to transition to value-based care, it is increasingly important to recognize the full value that gene therapies offer.

Some organizations have created assessment metrics that they and payers use to attempt to determine the value of health technologies. These current metrics do not account for the transformational promise of gene therapies. It is critical that we modernize value assessments to accommodate the unique nature of gene therapies.

IGT has worked with our Advisory Council members and other stakeholders to determine the most critical updates to current value assessments. The following elements will help ensure gene therapies unique value to patients, the healthcare system, and society, are considered:



#### DISEASE STATE FACTORS

- Burden on Patient
- Addressing an Unmet Medical or Clinical Need
- Severity and heterogeneity of disease
- Rarity of Disease
- Family & Caregiver Burden



# TREATMENT FACTORS

- Short-term and long-term treatment effects
- Lifetime Impact and Durability
- Patient Reported
  Outcomes



#### SYSTEM IMPACT

- Direct and indirect Medical Costs
- Decreased hospitalizations



# **SOCIETAL IMPACT**

- Advancing Health Equity
- Goodwill and the Value of Hope
- Giving Patients and Families More Time Together
- Extending Patients' Ability to Contribute to Communities

# Understanding the Value of Gene Therapies



Gene therapies are unique in both their potentially curative, durable outcomes for patients who may otherwise have no treatment options and in their development process. We believe the following principles are critical to any assessment of a gene therapy's value:



No public payer or private sector organization should place a dollar value on a patient's quality of life, health, and worth.



Determinations of value may be subject to change based upon evolving real-world evidence.



Value is just one component of a comprehensive and complex coverage decision process that prioritizes patient's medical needs and available treatment options.



One-size-fits-all value assessments are ineffective and discriminatory.



Value assessments should adapt and reflect the available evidence for rare and ultrarare disease treatments.



The patient, family, and caregiver perspective and preferences must be given priority.



Determinations of value should uphold FDA's scientific authority over drug approvals.



An informed healthcare assessment will incorporate the vital elements of value across the four domains highlighted above.

PATIENTS, FAMILIES, OUR HEALTHCARE SYSTEM, AND SOCIETY DESERVE A MODERNIZED VALUE FRAMEWORK.

# LEARN MORE AT GENE-THERAPIES.ORG