Patient-Centric Value Assessment Processes That Provide Timely and Sustainable Access and Incentives to Current and Future Therapies

⚠ PROBLEM:

Recent research found that rare disease therapy on average takes nearly four years longer to develop compared to non-rare therapies (11.8 years vs 8 years) at substantial cost and requiring higher pricing to ensure appropriate return on investment[1]. The heterogeneity and uncertainty around health technology assessment (HTA) requirements for clinical evidence, in addition to the higher costs associated with individual rare disease therapies, translates into challenges in assessing the value and the economic impact of rare disease treatments. Evaluation of value via "single yardstick" approaches are designed for more prevalent diseases, and where there is clinical consensus around the preferences and needs of patients[2]. When used for rare conditions, traditional HTAs may limit or preclude access for patients with substantial unmet need/limited or no treatment solutions[4].

Given that rare diseases tend to be complex and clinically heterogenous with multiple variants (stage-based/flare-based, relapsing, etc.) even within one small patients population, current approaches and assessment methodologies are ill-equipped to evaluate treatments for these types of diseases nor do they adequately or accurately reflect patient-centered value associated with treatments for rare diseases. Coverage and reimbursement decisions based on HTAs typically fail to account for real-world impacts, patient burdens, experiences, or outcomes for families, caregivers and patients when evaluating the value of new treatments and the improvement in quality of life that a new treatment could provide, especially in rare disease where little data on the patient experience and disease burden is available. And for patients suffering from a rare disease, quality of life is often significantly lower compared to patients who are otherwise healthy or have more common diseases[3,4].

SOLUTION:

- Consideration of different value drivers associated with common rare disease archetypes, (e.g., early/fast fatal, stage-based, flare-based, continuously progressing) and calibrate decision criteria accordingly.
- The development of a quantitative/qualitative approach to rigorously capturing the preferences of patients and the value that a specific patient community applies to certain improvements in symptoms and quality of life.
- Validate position with data and examples demonstrating how the current approach can harm patients, and why new approaches informed by patients and real-world evidence should be adopted. This type of approach is becoming more commonplace in the evaluation of treatments for cancers with small patient populations.
- Encourage competition in orphan drug development through the use of tax initiatives and other incentives (e.g., the Orphan Drug Act provides incentives that include clinical research grants, waived PDUFA fees, research and development tax credits for and a greater period of market exclusivity).

SOURCES

1. Tufts Center for the Study of Drug Development. Tufts CSDD Impact Report. July/August 2019. https://csdd.tufts.edu/impact-reports. Accessed June 2020.
2. Simoens S, Picavet E, Dooms M, Cassiman D, Morel T. Cost-effectiveness assessment of orphan drugs: a scientific and political conundrum. Appl Health Econ Health Policy. 2013;11(1):1-3.
3. Cohen JS, Biesecker BB. Quality of life in rare genetic conditions: a systematic review of the literature. Am J Med Genet A. 2010;152A(5):11361156.
4. Bogart KR, Irvin VL. Health-related quality of life among adults with diverse rare disorders. Orphanet J Rare Dis. 2017;12(1):177.
5. Acess to Critical Therapies White Paper https://globalgenes.org/resources/guiding-principles-of-rare-disease-care-and-patient-access/

ABOUT US

Global Genes is a 501(c)(3) non-profit organization dedicated to eliminating the burdens and challenges of rare diseases for patients and families globally. In pursuit of our mission we connect, empower, and inspire the rare disease community to stand up, stand out, and become more effective on their own behalf -- helping to spur innovation, meet essential needs, build capacity and knowledge, and drive progress within and across rare diseases. We serve the more than 400 million people around the globe and nearly 1 in 10 Americans affected by rare diseases. If you or someone you love has a rare disease or are searching for a diagnosis, contact Global Genes at 949-248-RARE or visit our resource hub at globalgenes.org.

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