A Patient-Centric Approach to Evaluate Rare Disease Treatments

Using a Holistic Assessment of Value to Drive Scientific Innovation Forward

A "one-size-fits-all" approach to drug pricing and value assessment frameworks undermines development of therapies for people – very often children - with rare diseases. As rare disease companies, we champion policies that help people with high unmet needs by developing innovative cutting-edge medicines and working with federal and state policymakers to address access and affordability.

Restoring Equity and Patient Focus when Capturing Value for Rare Disease Patients

- Traditional value-assessment frameworks emphasize value to the payer by prioritizing reduced costs over outcomes and are often designed to assess disease areas that have multiple treatments available. As a result, value frameworks will typically consider just two core inputs - Quality Adjusted Life Years (QALYs) gained and Net Cost.
- This approach presents shortcomings for assessing the value of rare disease therapies and if used to inform coverage decisions will limit patient access to treatments they need. Issues include:
    - QALY is a single measure that combines how much a treatment extends life and improves quality of life. This flawed measurement assigns a lower value to patients with disabilities or debilitating conditions than those without. QALYs devalue an elderly or disabled patient that is living with a rare disease, reinforcing inequities in our healthcare system. The National Council on Disability has recommended banning the use of the QALY as it violates the American Disabilities Act.
    - The Net Cost metric looks at value from a payer perspective, only incorporating the cost of current treatment paid for by the insurer. But for many rare diseases, costs incurred by the payer are often low, given limited availability of treatments. Direct costs to the patient are not quantitatively accounted for in traditional value frameworks (i.e. symptom management, lost wages from the patient and caregiver).

A Holistic, Patient-Centered Approach to Valuing Rare Disease Treatments Will Promote Access, Health Equity and Future Innovation

- Value-assessment must account for the rarity, severity and progression of the disease throughout a patient's life.
- Direct and indirect benefits of a treatment, particularly those most important to the patient, should also be incorporated into value assessments. Such inputs include the intervention's impact on symptom control, improvement over alternative treatments (if available) and compared to natural history, ability of a patient to participate in activities of daily living, impact on caregiver burden, impact on public health, benefits to the economy, and the value of hope.
- The impact of the therapy being assessed on future innovation — e.g., a basis for learning for subsequent development including in combination with therapies developed in the future — as well as the reduced cost of the therapy when generics become available should also be incorporated into the value assessment.

Policy Proposals Should Incentivize Rare Disease Treatment Development

- Millions of Americans living with rare diseases, 90% of them without FDA- approved therapies, maintain hope for new or better treatments that will improve their quality of life.
- While rare disease treatments are manufactured for limited population sizes, research and development costs often remain the same, if not higher due to the lack of natural history, complex diagnosis and limited access to people living with rare diseases.
- We applaud Congress and state legislatures' deliberate intent over the past three decades to incentivize rare disease drug development, and the Administration's current push for increased American innovation.
- To build on that progress, the development of alternative methods for value frameworks must be developed to accurately define how value is calculated for rare disease patients including encouraging real-world data, prioritizing patient outcomes over payer costs, valuing innovative trial design, advancing health equity, and rejecting foreign price controls.

For more information, please visit www.rarecoalition.com or contact info@rarecoalition.com. Follow us on Twitter at @RareCoalition.

Rare Disease Company Coalition

06_2022