Rare Disease
Company Coalition

Undervaluing Rare Disease Treatments Punishes Rare Disease Patients

Don't stifle innovation for those who need it most

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 Congress and State Legislators to address access and affordability.

Value Assessment Methods are Flawed in Capturing Value for People with Rare Diseases

- Existing value-assessment frameworks are designed to assess disease areas that have multiple treatments available.
- Value frameworks will typically consider just two core inputs - Quality Adjusted Life Years (QALYs) gained and Net Cost -which have shortcomings for assessing the value of rare disease therapies.
- For example, QALYs devalue an elderly or disabled patient that is living with a rare disease, reinforcing inequities in our healthcare system.
- In another example, the Net Cost metric used in existing value frameworks looks at value from a payer versus patient perspective even though the patient bears many costs that are accounted for in this measure (i.e. symptom management, lost wages).
- For many rare diseases, standard of care costs incurred by the payer are often low given limited availability of treatments. A new treatment, when there was none before, creates a new standard of care and could have a positive benefit for payers and patients if value is viewed in totality of the particular circumstances.
- Value-assessment tools must be patient-centered, incorporating patient perspectives, accounting for the treatment's impact on holistic patient experiences and should reflect not only population-level information, but also individual patient viewpoints and disease journeys.
- Additionally, value-assessment tools must consider both the direct and indirect benefits of a treatment, particularly those most important to the patient. For example, progression of disease, symptom control, a treatment's impact on productivity, ability of a patient to participate in activities of daily living, impact on caregiver burden, improvement over alternative treatments, impact on public health, and the value of hope.

#OneRareVoice

Policy Proposals Would Disincentivize Rare Disease Treatment Development

- Policy proposals allowing Medicare and Medicaid to adopt value frameworks to negotiate the price of drugs that have no competition in the market would block access to new treatments available for rare disease patients.
- The unmet need in rare diseases continues to be significant as only 7% of rare diseases have an FDA-approved treatment option, most of which have no therapeutic alternatives.
- These discriminatory models and proposals are also in direct opposition to 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.

Potential Impacts to Rare Disease Companies & Patients

- Millions of Americans living with rare diseases, most of them without FDA-approved therapies, maintain hope for new or better treatments that will improve their quality of life.
- Existing value assessments fail to account for the full spectrum of costs and benefits to patients and caregivers, thus compromising the hope that people living with rare diseases have by disincentivizing development and creating barriers to innovative therapies.
- Economic and quantitative analyses do not account for the lived experiences and perspectives of the small and dispersed population of rare disease patients, their caregivers or communities.
- Policies should account for the complexity of rare disease drug development, including that research and development for treatments require substantially different trial design and business models than therapies for larger patient populations.
- While rare disease treatments are manufactured for limited population sizes, research and development costs remain the same, if not higher due to the lack of natural history, complex diagnosis and limited access to people living with rare diseases.

What Policy Proposals Need to Consider

- Any step towards implementing a price-setting authority with a value framework risks irreparable harm on the thriving ecosystem of rare disease research, and will be compounded if extended into the commercial market.
- Adopting a "one-size-fits-all approach" to value assessments – one that neglects to quantify all components of value for rare disease patients – will ultimately undervalue rare disease treatments will ultimately undervalue rare disease treatment, inadvertently depriving those patients of innovative new therapies.
- The development of alternative methods for value frameworks should be grounded in principles that encourage real-world data, redefine how value is calculated for rare disease patients, prioritize patient outcomes over payer costs, value innovative trial design, advance health equity, and reject foreign price controls.

Rare Disease Company Coalition

We are dedicated to being a reliable and trusted resource for our leaders in Congress and the Administration, and a productive partner to rare disease-minded industry, academic and patient groups, by educating on the issues and opportunities that affect rare disease companies and advancing our shared mission to improve the lives of people living with rare diseases.

For more information, please visit www.rarecoalition.com or contact info@rarecoalition.com.
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