Rare Disease
Company Coalition

Existing Value Frameworks to Negotiate Pricing for Rare Disease Drugs Disproportionately Impacts People Suffering from Rare Diseases

Don't Stifle Innovation for Those Who Need it Most

A "one-size-fits-all" approach to drug pricing and value assessment frameworks will stifle the investments in and developments of new and improved medicines for people with rare diseases. As rare disease companies, we are committed to working with Congress to address patient affordability. However, a policy that targets the innovators bringing treatments to patients with high unmet needs will mean fewer cutting-edge medicines for the patients – very often children - who need them most.

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

- Existing value frameworks, including those relied upon by many countries outside the U.S., are rigid and force the evaluation of new innovative medicines in areas of high unmet needs into a framework that is more suited for chronic therapies where there are multiple treatments available for a given disease.
- Value frameworks will typically consider just two core inputs - Quality Adjusted Life Years (QALYs) gained and Net Cost -which have shortcomings for assessing rare disease therapies.
- For example, QALYs devalues an elderly or disabled patient that is living with a rare disease, reinforcing inequities in our healthcare system.
- For many rare diseases, standard of care costs incurred by the payer are often low given limited availability of treatments. 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.
- A more accurate evaluation of a therapy used for treating rare diseases must account for multiple other factors - including productivity, severity of disease, reduction in uncertainty, patient and caregiver burden, equity, society preferences and scientific spillover - that are not captured in existing value frameworks.

Policy Proposals Would Disincentivize Rare Disease Treatment Development

- Policy proposals allowing Medicare to adopt value frameworks to negotiate the price of drugs that have no competition in the market would disproportionately impact 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.
- The proposals are also in direct opposition to Congress' deliberate intent over the past three decades to incentivize rare disease drug development and the Administration's current push for increased American innovation.

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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.
- Recent policy proposals ignore 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 economies of scale are unfavorable, 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 and punish rare disease patients.
- The development of alternative methods for value frameworks should be grounded in principles that encourage real-world data, redefine what value means for rare disease patients, prioritize patient outcomes over payer costs, value innovative trial design, advance health equity, and reject foreign price controls.
- The Administration and policymakers must recognize the potential for investment in rare diseases to dry up, and the likely impacts to the biotech ecosystem across development programs, partnerships, and the commercialization processes.