COMMUNITY CONGRESS

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Rare Disease Community Statement on Drug Pricing Policy Priorities

Introduction

Over 30 million Americans live with one or more rare diseases that often result in burdensome medical, indirect and non-medical expenses. Patients and families are left to navigate how to manage expenses from multiple inpatient and outpatient encounters, costs for prescription therapies and medical devices, and the support services that are critical for managing their health and wellbeing.

The National Economic Burden of Rare Disease Study in the United States estimated in 2019 the overall annual economic burden of rare disease exceeded $966 billion. Of the total economic burden, the largest costs were indirect costs from productivity losses at $437 billion, direct medical costs at $418 billion and non-medical and uncovered healthcare costs of $111 billion absorbed directly by families living with rare diseases. Aside from absenteeism, inpatient care was the biggest expense, accounting for nearly 15% of the overall economic burden while prescription medication and administration costs accounted for about 10% and outpatient care for about 6%. Meaningful policy engagements around affordability must focus on more than just one aspect of the overall health care system[1].

Lowering the cost of health care, including prescription drug therapies, is an important but nuanced goal of multiple legislative proposals under consideration in Congress and state legislatures. As elected officials consider different policy options, they must recognize the unique complexities of rare disease drug development and the high unmet need faced by the more than 30 million Americans living with rare diseases[2].

While exciting advances in science and medicine have propelled new rare disease therapies into reality, between 93-95% of the over 7,000 rare diseases still have no FDA approved treatment. For those living without approved treatments, policies intended to lower drug costs without consideration of the impact on incentives for rare disease research and development infrastructure will mean lives are lost and unnecessary suffering will be extended.

It is imperative that policymakers understand why rare disease research and development is more challenging, time consuming and costly than non-orphan products despite existing incentives such as those created by the Orphan Drug Act. To qualify as a rare disease, a condition must affect less than 200,000[3] in the U.S., however in many cases, innovative therapies are being developed for diseases with much smaller populations, sometimes even as few as 12 patients. The trend toward smaller patient populations will likely only continue as diseases are better understood and therapies targeted for genetic subsets of a disease. Clinical development programs for very small populations require extensive investment in order to generate enough evidence to understand the natural history of the diseases, to overcome diagnostic uncertainty and to validate outcome measures among other factors.

The time it takes to conduct clinical trials for rare disease products is twice that of non-orphan products[4] all while the risks of failure are higher.

Any policy solutions that move forward must be patient-centered and focused on improving the access environment for rare disease therapies while not resulting in unintended harm to the delicate research and development ecosystem. When governments set the price of medications, medical innovation slows, and patients lose access to lifesaving treatments. The extended delays on medicine access could mean the difference between life and death for American patients in need of treatments. Other countries have delayed access to innovative treatments due to the price controls imposed by their governments, rare disease patients in the U.S. cannot be subject to these delays.

The principles outlined below have been developed in collaboration with the rare disease community to convey the goals that any proposed drug pricing policies should be evaluated against.

Principles to Guide Drug Pricing Policy

Incentivize Rare Disease Therapeutic Development

- Policy makers should recognize that therapies for rare diseases are developed for areas of high unmet need and have unique complexities that make policy solutions developed for therapies to treat more prevalent not always appropriate for rare diseases.
- Preserve and protect existing incentives for rare disease drug development.
- Seek new ways to incentivize the development and repurposing of treatments and cures for rare diseases.
- Avoid pricing and reimbursement policies that would disincentivize the use of existing FDA expedited approval pathways.
- Seek policy solutions that prevent abuses of rare disease incentives to ensure such incentives are utilized to bring therapies to rare disease patients as originally intended by the Orphan Drug Act.

Uphold the Role of the FDA

- Ensure that all policy solutions include an assurance of scientific and regulatory rigor such that rare disease therapies are safe, effective and affordable.
- Policy solutions should recognize FDA's statutory authority in determining a medical product's safety and effectiveness and promote timely access to approved therapies.

Innovations in Payment Models

- Generate evidence to support disruptive innovation in the models used to bring breakthrough therapies to patients at lower costs while not harming future medical innovation.
- Seek new and alternative payment models, including outcomes-based contracting, that allow patients to have access to novel therapies and ensure that reimbursement policies encourage development of future curative therapies.

- Exclude rare disease therapies from policy experiments or demonstrations that significantly alter how prices are set until or unless it is proven that the changes do not threaten patient access and medical innovation.

Put Patients at the Center of Defining Value

- The quality adjusted life year (QALY) and similar discriminatory measures must not be included when determining cost-effectiveness or informing reimbursement and payment models.
- Advance policies that recognize the full value of a rare disease treatment by incorporating meaningful inputs from patients and scientific perspectives that traditional value frameworks exclude, including the generally higher burden of disease on patients with rare diseases.

Decrease the Patient Access Hurdles of Time and Cost

- Ensure policy solutions better align patient access to time of approval rather than imposing further process delays, especially for diseases that are irreversibly and progressively debilitating.
- Seek policy solutions to decrease burdensome utilization management barriers which lead to delays in patients accessing therapies.
- Coverage and reimbursement policies should ensure patients have access to appropriate FDA approved therapies in consult with expert healthcare providers.
- Seek policies that create transparency in the healthcare system or in how pricing decisions and access determinations are made.
- Policy solutions should seek to lower overall out-of-pocket costs for patients and families and spread costs over the annual plan term.

Many of the transformational therapies that are approved face access barriers that prohibit or delay these life-changing treatments from reaching patients. This counteracts the benefits of expedited pathways that speed the availability of drugs to treat serious diseases that are intended to ameliorate unmet patient needs and reduce long term costs by allowing for early access to innovative treatments.

Further, utilization management techniques are employed by public and private payers in order to limit their expenses, but these policies have the effect of increased costs for patients and often irreversible disease progression and loss of life. In addition to utilization management techniques, value assessments are increasingly being used by payers as a tool to support limited product access. While the use of value assessments and innovative value-based payment arrangements may be one way to understand a product's value within a patient subpopulation, it should not be used as a singular tool to control costs. It is also important to note that current value assessments conducted of rare disease therapies are not based on frameworks and models that enable accurate assessments of value. Limitations include that calculation of the Quality-Adjusted Life Year (QALY) gained is widely considered to be discriminatory towards patients with disabilities, patient heterogeneity and subgroup analyses are often not considered, and additional elements of value afforded by innovative therapies, such as increased productivity and reduction of caregiver burden, are often not included.

Congress has already recognized there are differences in the resources required to develop a new treatment for a rare disease through their work on the Orphan Drug Act, 21st Century Cures and more.

We urge Congress and all those policymakers working on drug pricing policies to once again consider the extensive unmet needs and scientific challenges inherent in the rare disease community and ensure policy proposals adhere to the principles developed by the collective rare disease community.

Sources

1. The EveryLife Foundation. The National Economic Burden of Rare Disease. www.burdenstudy.org. Accessed May 4, 2021.

2. U.S. Food and Drug Administration – Rare Diseases at FDA. https://www.fda.gov/patients/rare-diseases-fda. Accessed May 4, 2021.

3. National Center for Advancing Translational Sciences–Genetic and Rare Diseases Information Center. Diseases. https://rarediseases.info.nih.gov/diseases. Accessed May 4, 2021.

4. Jayasundara, K., Hollis, A., Krahn, M. *et al.* Estimating the clinical cost of drug development for orphan versus non-orphan drugs. *Orphanet J Rare Dis* **14,** 12 (2019). https://doi.org/10.1186/s13023-018-0990-4