Rare Disease Patient Organizations Statement on Drug Pricing Proposals

As Congress continues to consider ways to address the affordability and pricing of emerging drug therapies, we encourage policy makers to remember that millions of Americans living with rare diseases do not have any access to treatments. This lack of access is not simply because they are unaffordable, but because the grim reality is that 93% of the 7000 rare diseases have no FDA approved therapies. Ensuring a favorable access environment is a priority for the rare disease community but must be approached in a manner that recognizes the complexities of the issue, while continuing to incentivize innovation within rare diseases.

The high cost and high risk of developing lifesaving therapies combined with small patient populations means the costs for rare disease treatments may often be higher per patient than treatments for larger patient populations. However, spending growth in rare diseases has been moderate over the last decade despite a higher volume of treatments, and rare disease treatments still represent a small share of overall prescription drug spending\(^1\). Biopharmaceutical companies must be able to demonstrate the potential for return on investment for rare disease therapies or they may not be developed. Once these therapies are developed, ensuring access to them is critical both for patients and for driving investment in the next generation of therapies.

The rising costs of healthcare disproportionally burden rare disease patients; however, the cost of drug therapies is only a portion of the financial burden. In the absence of effective treatments and timely diagnoses, rare disease patients must incur the costs of extensive diagnostic odysseys, frequent hospitalizations and emergency room visits, travel to disease specialists, invasive tests and surgeries, respiratory support, durable medical equipment, weekly therapies and nursing care. In addition, caregiver support often requires that a spouse or parent also leave the work place, and additional services be provided through schools and community services where costs are incurred not only by the individual families but also public and private programs. Furthermore, many rare disease patients utilize off-label treatments that are usually not covered by insurance.

Given the complexities of rare disease innovation and access, we ask that Congress work toward developing proposals that continue to incentivize innovation within rare diseases. Therefore, we encourage Congress to:

- Preserve and protect existing incentives for rare disease drug development.
- Find new ways to incentivize the development and repurposing of treatments and cures for rare disease patient populations.
- Exempt orphan products for life threatening rare disease from any drug pricing or reimbursement regulation that could stifle innovation or prevent a company from continuing to market rare disease products.
- Seek policy solutions that create transparency in the healthcare system.
- Encourage policy solutions that prevent abuses of rare disease incentives to ensure that incentives are utilized to bring therapies to rare disease patients as originally intended by the Orphan Drug Act.

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\(^1\) IQVIA. “Orphan Drugs in the United States: Growth Trends in Rare Disease Treatments.” October 2018.
• Seek policy solutions that encourage competition for generic and branded therapies to ensure a fair and equitable marketplace.
• Seek new and alternative payment models that allow patients to have access to novel therapies and ensure that reimbursement policies encourage development of future curative therapies.
• Ensure that all policy solutions include an assurance of scientific and regulatory rigor such that rare disease therapies are safe, effective and affordable.

We look forward to working with Congress to help accelerate the development of novel cost-effective therapies and to ensure accessibility of these treatments for all rare disease patients.

**Endorsing Organizations:**
EveryLife Foundation for Rare Diseases
Pemphigus Pemphigoid Foundation
Noah's Hope-Hope4Bridget Foundation
American Behcet's Disease Association (ABDA)
APS Foundation of America, Inc.
Parent Project Muscular Dystrophy (PPMD)
Gaucher Community Alliance
MAGIC Foundation
Cutaneous Lymphoma Foundation
Fibrous Dysplasia Foundation
Rare New England
Hypersomnia Foundation
Sick Cells
National MPS Society
Association for Creatine Deficiencies
Dandy-Walker Alliance, Inc.