



### **Rare Access Action Statement on HR 3**

On April 22<sup>nd</sup>, 2021, the House reintroduced [Elijah E. Cummings Lower Drug Costs Now Act](#) (H.R. 3) to address the affordability of drugs and biologicals in the United States, which includes the same redesign of the Medicare Part D benefit that caps beneficiary out-of-pocket costs and shifts a sizeable percentage of those costs back on to the manufacturer. Although we recognize the importance of capping out of pocket patient expenses for rare disease patients, we must weigh those benefits with the risks to the fragile research and development incentives for rare and orphan diseases. Not all drug markets are the same.

**90% of Orphan Disease Have No Approved Treatment.** Despite great progress over the last couple of decades as a result of the bipartisan commitment to finding treatments for rare diseases, as evidenced by the Orphan Drug Act (ODA), more than ninety percent (90%) of the identified 7,000 rare and orphan diseases [have no FDA approved therapies](#).<sup>1</sup> We have made so much progress, but we have so much further to go. Rare disease research and development must be protected.

Rare/orphan disease is defined as affecting less than [200,000 people in the US](#), but the populations with specific rare diseases can often be much smaller (known as ultra-orphan diseases) particularly as we see advancements in genetic profiling of diseases. The Part D benefit redesign in H.R. 3 presents significant risks to the fragile landscape of rare disease research and development for rare patient populations; caps on out-of-pocket expenditures are of no benefit to rare disease patients if there are no treatments for rare diseases.

**Defining the Risk.** An analysis conducted by [Xcenda](#)<sup>2</sup> illustrates the profound and disproportional risk of the H.R. 3 Part D benefit redesign proposal on the rare disease market. The analysis found that modeling for a few rare disease treatments show that the manufacturer obligations are projected to rise from 1,000% to 2,000%. In comparison, the estimated increased liability for a chronic disease, Type 2 diabetes product is 29%. Such a significant increase in manufacturer liability for rare disease products would destroy the already fragile incentives for rare and orphan disease research and development particularly when the innovators are more often a small and emerging companies.

**Unique Challenges in Research and Development in Rare Diseases.** A Tufts Center for the Study of Drug Development study published in the Journal of Health Economics concluded the cost of developing a new drug can go as high as \$2.6 billion, and only about 12 percent of those under development succeed.<sup>3</sup> In rare disease

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<sup>1</sup> National Organization for Rare Disorders (NORD), "New Study Investigates the Number of Available Orphan Products, Generics and Biosimilars." <https://rarediseases.org/new-study-investigates-the-number-of-available-orphan-products-generics-and-biosimilars/>. Accessed April 23, 2021.

<sup>2</sup> Xcenda and RAAP. "Changes in Medicare Part D Benefit Design Can Impact Availability of Future Treatments." 2020. <https://www.xcenda.com/-/media/assets/xcenda/english/content-assets/white-papers-issue-briefs-studies-pdf/raap-part-d-redesign-impact-on-rare-disease-manufacturers-feb-20.pdf?la=en&hash=F3D17C96BAF109F70B69A06EA58327A952EC9A9C>. Accessed April 26, 2020. Accessed April 25, 2021.

<sup>3</sup> Sullivan, Thomas. "A Tough Road: Cost to Develop One New Drug Is \$2.6 Billion; Approval Rate for Drugs Entering Clinical Development Is Less Than 12%." *Policy & Medicine*, March 2019.



research, costs vary widely and clinical trials present additional unique challenges, including ill-defined and very small patient populations, lack of natural histories, and few assays or diagnostic tests.

**Protections for Rare Disease Research and Development.** Uncertain return on investment for rare disease therapies keeps many companies out of the market. Despite these challenges, a few intrepid emerging companies are forging ahead with new treatments for diseases that have languished by convincing financial stakeholders to take a leap of faith, even in the face of uncertain markets and hard to find patient populations. The broad policies of H.R. 3 targeting do not consider the dynamics of the unique markets. In particular the significant unmet medical need, the size of the orphan/rare disease populations, and the unique research and development costs particularly in rare and even more so, ultra-rare orphan diseases places continued success in bringing rare disease treatments to market at significant risk.

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**Rare Access Action Project (RAAP) Advocating for Rare Disease Access.** The Rare Access Action Project ([RAAP](#)) is a policy coalition of rare disease advocates, emerging life sciences companies, and other rare disease stakeholders that seek innovative solutions to barriers that hinder rare disease patient access to therapies. RAAP is committed to exploring policy solutions to address structural issues in access and coverage for patients with rare diseases. While we recognize the intricate dynamics of drug pricing and the relationship to patient access, we also understand the importance of thoughtful policy changes that protect development of innovation along with patient access to that innovation, particularly in the case of rare diseases.

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*My family is fortunate my 15-month-old son was diagnosed in the hospital after a nightmarish few weeks of emergency rooms, specialists, blood draws, and sleepless nights by his hospital bedside with a rare disease. After diagnosis, months of daily injections of a biological saved his life, and by extension my life and I am eternally thankful. More importantly, I know how fortunate we are, there was a treatment for his orphan disease. I know it is by the grace of God that his treatment was brought to market. The biological that saved my son's life was not so effective for the originally intended target a much, much larger market. Fortunately, the product was not shelved as so often can happen given more profitable research priorities but rather the product was still brought to market and has been available for this orphan population. We were so lucky, and the "what if's" often haunt me 6 years later as I watch my healthy son excel at soccer. Research and development in the orphan market are fragile. We must preserve every incentive for research and development to treat rare, orphan diseases.*

Rare Disease (sJIA) Mom  
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