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March 15, 2024

Congresswoman Virginia Foxx
Chairwoman
U.S. House of Representatives Committee on Education and Workforce
Washington, D.C. 20515

Dear Chairwoman Foxx,

On behalf of patients and families affected by rare diseases, the EveryLife Foundation for Rare Diseases is pleased to offer the following comments to inform the House Committee on Education and Workforce's efforts to modernize and strengthen the Employee Retirement Income Security Act (ERISA).

The EveryLife Foundation for Rare Diseases is a 501 (c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures. EveryLife's establishment of the Community Congress, a diverse coalition comprised of patient advocacy organizations, industry leaders, and other relevant stakeholders, guides our policy efforts and provides advice and insight on urgent policy issues impacting the rare disease community.

Over 30 million Americans live with one or more rare diseases, 95% of which have no approved treatment. Rare disease patients and families 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 their health and wellbeing. The National Economic Burden of Rare Disease Study estimated that in 2019, the overall economic impact of rare disease in the U.S. exceeded $966 billion[1]. Of the this, 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[2].

Nearly 60% of the overall cost of living with a rare disease in the US are costs being absorbed directly by families living with rare diseases, employers, and society at large[3]. Meaningful policy engagements around affordability must focus on more than one aspect of the overall health care system, including transparent and comprehensive health care coverage.

We are thankful to the Committee for your anticipatory approach and for seeking the expertise and insights of the community as you work to understand how current ERISA policies impact access to affordable and quality coverage

[1] Yang, G., Cintina, I., Pariser, A. et al. The national economic burden of rare disease in the United States in 2019. Orphanet J Rare Dis 17, 163 (2022). https://doi.org/10.1186/s13023-022-02299-5
[2] Ibid
[3] Ibid

and what policy innovations might improve access moving forward. While we recognize the intended audience for your RFI is employers, the insights of patients, families and the organizations that represent them are essential to fully understand the impact of policies that govern self-funded plans. We are pleased to offer the following insights on the impact of ERISA policies on the rare disease community.

Overarching Concerns

For many in the rare disease community, the availability of an approved treatment is still out of reach. As we celebrate progress in scientific discovery and therapy development, we recognize that for the 5% of rare diseases that do have a treatment approved, challenges in navigating the access environment take over. Additionally, most rare disease patients struggle to navigate a system that is not designed to account for the complexity of managing rare diseases.

While some of the challenges expressed throughout this response aren't specific to ERISA plans, they are often magnified by the lack of consistent information and consumer protections available to patients and families in public and fully insured plans. Improvements in transparency and oversight of ERISA plans were common threads throughout our Community Congress discussions to inform this response. Employees with a rare disease or a covered family member with a rare disease struggle to obtain clear information on covered services and treatments, what rules apply to self-funded plans like theirs, what appeal rights they have, how to expedite issues of concern, and how to ensure that a qualified individual is making determinations about the type of care and expertise they can access. While these are issues regardless of the rarity of one's disease, they are magnified for those with complex rare diseases that are generally not represented in policy and communications decisions.

As the Committee considers ERISA reforms that speak to transparency and appeal requirements within self-funded plans, consider that for many with a rare disease or a covered dependent with a rare disease, fear of retaliation or discriminatory practices because of speaking up, may lead to these employees suffering in silence.

Coverage Barriers

As rare disease therapy development progresses, we recognize the challenges employers face paying for treatments that carry high upfront costs and accrue long-term cost savings. Unfortunately, the solutions that employers have deployed to create savings result in adverse health outcomes, exacerbate stress and anxiety, and do nothing to address the longer-term needs of an employee or covered family member.

Alternative Funding Plans

Increasingly, employers are contracting with third party vendors known as Alternative Funding Programs (AFPs) that claim to create significant savings through managing access to certain specialty drugs. By advising health plans to exclude coverage for specialty drugs, AFPs are creating the fraudulent appearance of noncoverage, some relying on a claim that specialty drugs are considered "non-essential health benefits."

In many cases, patients receive an abrupt notification that their medication is no longer covered and are told: (1) that their drug will not be covered unless they cooperate with the AFP vendor and apply for coverage through the patient assistance program (PAP)(i.e. "If you do not participate in the program, you will have a 100% reduction in your payable benefit for specialty medication."); and (2) that if

coverage is unsuccessful through the PAP, their employer will reinstate their coverage, though in some cases they are simply left without access to their necessary treatment.

Patients are thus forced by the health plan and AFP to participate in deceiving the PAP by claiming that they are without coverage even if coverage from their plan remains available. In many instances, this means consumers are forced to choose between violating the terms and conditions of enrollment in a PAP or paying entirely out of pocket for costly medications. This puts patients, through no fault of their own, in an untenable position.

If the AFPs eventually secure free drug through the PAP after a prolonged delay, the limited availability of PAP funding means the patient will have to overcome hurdles to timely treatment access again once the assistance is depleted. These challenges are experienced by patients newly initiating a treatment and those on long-term therapy for serious, chronic, and often rare conditions. The drug is not excluded for medical reasons – but instead for the sole purpose of shifting financial responsibility to the charitable PAP. This notification is not only distressing for consumers but can result in arbitrary delays as consumers wait for the PAP to determine whether they are eligible for the program. In addition to delays, other patient impacts include a lack of coverage for the supplies and services needed to administer the drug, which PAP programs do not provide.

Alternative Funding Plans threaten to dismantle the promise of comprehensive drug coverage for many Americans, particularly those who rely on specialty drugs such as the rare disease community. A task force of patient advocacy organizations has held multiple meetings with the Department of Labor and other relevant agencies to inform them of these challenges and urge them to take all available enforcement action to stop this deceitful and discriminatory practice. This task force, including the EveryLife Foundation, sent a separate and robust response to your RFI detailing concerns about AFPs and other related issues and we strongly support the recommendations laid out in that communication.

Other Utilization Management

In rare diseases, time is a precious commodity. While we appreciate the challenges of providing comprehensive coverage for specialty medications, increased reliance on inappropriate utilization management techniques creates unnecessary hurdles to critical and time-sensitive treatment interventions, yielding irreversible disease progression and catastrophic healthcare costs. In 2021, 94% of prior authorization requests with Medicare Advantage plans were approved, but of the over 2 million requests that were denied, only 11% were appealed while appeals were met with an 80% success rate[4]. The data aligns with the stories we consistently hear from rare disease patients. While it is understandable that some level of utilization management is medically appropriate, it must occur in a timely and transparent manner to ensure access to treatments that so many in the rare community have been searching for years to find.

EveryLife supports policies that will ensure utilization management policies are medically appropriate and create expediting decision and appeals processes. The Safe Step Act (H.R. 2630) will address step therapy, also known as “fail first,” a practice that forces patients to try

[4] Biniek, J. F., & Sroczynski, N. (2023, February 2). Over 35 million prior authorization requests were submitted to Medicare Advantage Plans in 2021. KFF. https://www.kff.org/medicare/issue-brief/over-35-million-prior-authorization-requests-were-submitted-to-medicare-advantage-plans-in-2021/

medications preferred by the insurer before approving coverage for the medication the doctor originally prescribed. **Passing the Safe Step Act will ensure employer health plans offer a medically reasonable and expedient step therapy exceptions process.**

Restrictive Coverage Criteria

The rare disease community is concerned about trends in employer-sponsored, public, and commercial insurance markets that restrict or deny access to innovative treatments. Two areas of particular concern include payers' labeling some innovative therapies as experimental or otherwise uncovered due to the use of the accelerated approval pathway, and payers' increasing likelihood to restrict coverage to the narrow population included in the clinical trial, despite a broader approved label.

Accelerated Approval
Unfortunately, many self-funded plans categorize drugs approved through the FDA's accelerated approval pathway as "experimental or investigational" in issuing negative coverage determinations. Therapies approved under the accelerated approval pathway are no longer experimental, having been evaluated via the same rigorous standard of safety and efficacy as products assessed via the traditional approval pathway. The distinction is in the type of endpoint evaluated for safety and efficacy. The accelerated approval pathway is predicated on an evaluation of efficacy based on a surrogate endpoint that is reasonably likely to predict clinical benefit to ensure that patients with serious and life-threatening diseases can access treatments while the expected clinical benefit is confirmed. For rare diseases that involve very small patient populations and heterogeneous presentation among other complicating factors, the approval of treatments based on surrogate endpoints is essential. These misguided coverage policies create heartbreaking access barriers for patients and families who have previously only known the pain that comes with having a rare disease for which there is no treatment, but now must cope with there being a breakthrough that they simply cannot benefit from. Payers, including self-insured plans, should not be allowed to base coverage policies on the pathway that was used to obtain FDA approval.

Indicated Population
Community Congress discussions also emphasized the access challenges patients face due to overly restrictive coverage criteria, an issue largely reported in self-funded plans. As innovative therapies are approved, more and more coverage policies create narrow eligibility requirements based on the clinical trial inclusion and exclusion criteria, rather than allowing access to the population indicated on the FDA approved label. Tufts Center for the Evaluation of Value and Risks in Health recently found that "338 (28%) of plan's Accelerated Approval drug coverage policies included restrictions beyond the FDA label," an issue that occurred more in non-oncology treatments like rare diseases.[5] More specific to rare diseases, this same work found that for "five non-oncology drugs, plans imposed restrictions beyond the FDA label indication 100% of the time"[6].

The FDA evaluates the totality of evidence supporting the approval of a therapy to determine the eligible population included in the label. While in some cases, the label could theoretically match the

[5] LaMountain, F., Beinfeld, M. T., & Chambers, J. D. (2024, March 14). Despite criticisms of accelerated approval pathway-commercial payers defer to FDA. Center for the Evaluation of Value and Risk in Health. https://cevr.tuftsmedicalcenter.org/news/despite-criticisms-of-accelerated-approval-pathway-commercial-payers-defer-to-fdac
[6] Ibid

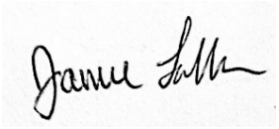
narrow group included in the clinical trial, in most cases, labels more closely reflect the real-world population likely to benefit from the therapy. By restricting coverage to clinical trial requirements, payers are usurping the role of the FDA and doing so without the benefit of the expertise and rigor that the FDA has applied to the process.

Out of Network and Out of State Care
Many individuals and families with rare diseases are required to travel, often out of state, to receive the healthcare they need. An EveryLife Foundation study found that on average, rare disease patients took 2.4 out-of-state trips and 17 different healthcare encounters just to obtain a diagnosis, contributing to average avoidable costs of $220,000 per person[7]. As of 2019, 39% of rare disease patients must travel over 60 minutes to access care, meanwhile about 17% of the respondents noted permanently relocating to access expert care[8]. As the Committee considers ERISA updates, it should seek to understand how employers are facilitating timely and affordable access to necessary specialists regardless of in-network status, especially in cases where no adequate expertise exists within the network.

Conclusion
The EveryLife Foundation eagerly anticipates further consideration on ERISA reforms that can optimize access to affordable and high-quality care and treatments, including specialty drugs. We welcome the opportunity for direct engagement with the Committee as we collectively enhance and advance policies that will improve the health and wellbeing of the 30 million Americans living with rare diseases.

Thank you for the chance to comment and for your commitment to addressing the unmet needs of the rare disease population. If we can provide additional information to support your process, please contact Jamie Sullivan, VP of Policy, at jsullivan@everylifefoundation.org.

Sincerely,



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[7] https://everylifefoundation.org/wp-content/uploads/2023/09/EveryLife-Cost-of-Delayed-Diagnosis-in-Rare-Disease_Final-Full-Study-Report_0914223.pdf
[8] The National Organization for Rare Disorders. November 2020. Barriers To Rare Disease Diagnosis Care And Treatment In The US: A 30-Year Comparative Analysis.