March 15, 2024

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

Dear Chairwoman Foxx,

In service of the neuromuscular disease (NMD) community, the Muscular Dystrophy Association (MDA) thanks Representative Virginia Foxx and The Committee on Education and the Workforce for the opportunity to comment on the Committee's Request for Information (RFI) regarding the Employee Retirement Income Security Act (ERISA).

MDA is the #1 voluntary health organization in the United States for people living with muscular dystrophy, ALS, and related neuromuscular diseases. For over 70 years, MDA has led the way in accelerating research, advancing care, and advocating for the support of our community. MDA's mission is to empower the people we serve to live longer, more independent lives.

Many of the therapies and treatments for neuromuscular diseases (NMD) would be unattainably expensive for the NMD community without insurance, and even with insurance, these burdens remain high. The National Economic Burden of Rare Disease Study estimated that in 2019 the overall annual economic burden of rare disease in the U.S. exceeded $966 billion,[1] Notably, that economic burden includes direct medical costs of $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 and the community.[3] In light of these expenses, access to quality insurance is vital for the NMD community to receive access to timely and effective care.

While we appreciate that the bulk of your questions about how ERISA can be improved are directed at employers, the beneficiary perspective, and particularly the perspective of some of the most vulnerable beneficiaries, is crucial to ensuring that ERISA provides effective coverage for all patients. To that end, we would like to flag two major areas to consider based on the Chairwoman's questions, first, the role an employer's fiduciary responsibilities play in ensuring access to robust and effective coverage, and second, the coverage of specialty drugs under ERISA as it applies to the rare disease community. Please see below for our answers to some of Chairwoman Foxx's questions from the patient advocacy perspective.

[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] Id.
[3] Id.

The Role of the Employer as Fiduciary in Effective Health Insurance:

Under ERISA, the fiduciary role serves as a key safeguard, ensuring that employers, and therefore their employees, receive the most robust care possible at the best price. Unfortunately, given the rise of our vertically integrated healthcare system and of Pharmacy Benefit Managers (PBMs), this duty has become increasingly difficult to manage.

A fiduciary's responsibility under ERISA is similar to that found in the law of estates. Put simply, fiduciaries must act prudently and for the exclusive benefit of participants in the plan when they select service providers for the plan.[4] "Implicit in a trustee's fiduciary duties is a duty to be cost-conscious."[5] In spite of this duty, however, plan fiduciaries' reliance on PBMs has made a marked negative impact on drug prices, which are most keenly felt by plan beneficiaries. PBMs offer various services to prescription drug plans, including negotiating with pharmacies to establish pharmacy networks where plan beneficiaries can obtain prescription drugs, helping manage plans' formularies, processing beneficiaries' claims in real time, and contracting with drug manufacturers to secure price reductions or other financial considerations. Here, PBMs break out into two models, a traditional model, and a pass-through model. Under a traditional model, the prices that a prescription-drug plan pays for prescription drugs are determined in negotiations between plan fiduciaries and the PBM and this negotiation can be as broad or as narrow as the fiduciary decides is prudent.

The price the plan agrees to pay its traditional PBM for a prescription may not bear any relation to the price the PBM will pay the pharmacy for the same prescription. The difference between these two price points, and i.e., what a traditional PBM takes as profit, is known as spread pricing. PBMs that engage in spread pricing are financially motivated not to make formulary decisions based on which drugs have the lowest cost to the plan and beneficiaries but rather based on which drugs allow them to pocket the largest spread. Prudent fiduciaries, therefore, closely supervise their formularies and carefully negotiate their payment structures to ensure that PBMs are not acting based on considerations that run contrary to the interests of the plan and its beneficiaries.

Some traditional PBMs also earn revenue through ownership of their own pharmacies. Express Scripts, for example, is a PBM that is vertically integrated with the specialty pharmacy. PBMs may then attempt to draw fiduciaries into using their vertically integrated pharmacies by charging higher formulary rates to pharmacies not in the vertical structure or by manipulating coverage of therapies not found in their vertically integrated pharmacies. This "steering" then, if not carefully monitored by the fiduciary will then be passed onto the patient (beneficiary) if or when they attempt to fill these prescriptions not using the PBM's selected pharmacies.

For all of the ways in which a PBM could seek to make a profit from fiduciaries at the expense of patients (their beneficiaries), ERISA, beyond its articulation of broad fiduciary duty, is silent as to any standard of what prudence means in light of these highly integrated systems, largely

[4] 29 U.S.C. § 1104(a)
[5] Rest.3d Trusts, § 88

unconsidered when ERISA was drafted. We would thank the Chairwoman for considering how this fiduciary duty should be updated going forward.

Specialty Drug Coverage:

On the issue of specialty drug coverage there are two major issues in the context of ERISA. First, the outright coverage of these therapies and the potential to consider risk pooling and other cost-saving measures therein, and second the current landscape of the cost of specialty drug tiers, again implicating PBMs.

Possibly the largest challenge employers face in offering coverage of high-cost specialty drugs is understanding that therapies approved under the accelerated approval pipeline are, in fact, fully FDA-approved and inherently medically necessary as they treat severe, unmet medical needs. In the last year, we've seen delays in care for Elevydis, Exondys 51, and Viltepso, among others.[1] While an approved therapy may slow or stop a patient's progression, the technology currently does not exist to reverse what a patient has lost while waiting for appropriate care. Between the progressive nature of NMDs and the small number of approved therapies, those living with an NMD are left with the choice of paying for these costly therapies out of pocket or waiting for the approval process to resolve. Regardless, while patients wait, their condition continues to progress. This then leads patients to bear the burden of navigating insurers' costly and time-consuming appeals process. If insurers better understand the utility of drugs approved via accelerated approval, then the employers who offer these plans would face less burden in negotiating with insurers as they make coverage and formulary decisions.

Similarly, we echo the Everylife Foundation's concern that, along with a lack of understanding and coverage for therapies approved via the accelerated approval pipeline, self-funded plans are often not covering approved therapies to the full label; "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."

Similar to the issue of coverage is the cost that is often passed on to patients using specialty drugs by PBMs., As originally envisioned, the "specialty" designation referred to expensive branded drugs used to treat complex or rare chronic conditions, required special handling or care.[6] Today, however, this designation has become watered down, by PBMs' consideration of what a specialty drug is. In fact, the three largest PBMs disagree about whether any particular drug is a "specialty" drug about 50% of the time.[7] In spite of this lack of certainty projected by PBMs, the largest PBMs, again, vertically integrate "specialty pharmacies" to manage the cost and dissemination of specialty drugs. If plan fiduciaries are not cautious when negotiating

[6] Patel, B. N., & Audet, P. R. (2014). A review of approaches for the management of specialty pharmaceuticals in the United States. *PharmacoEconomics*, *32*(11), 1105–1114. https://doi.org/10.1007/s40273-014-0196-0

[7] This analysis was conducted by 46 Brooklyn who utilized Elsevier Gold Standard Drug Database farming from CVS, Aetna, Cigna, Express Scripts, and Optum/United Healthcare. 46 Brooklyn's report is here: https://www.46brooklyn.com/research/2023/5/10/how-pbms-distort-and-undermine-specialty-drug-pricing-guarantees-blac and the various insurance databases are linked as follows: *(CVS / Aetna, Cigna / Express Scripts, and Optum / UnitedHealthcare)*

specialty provisions specifically, it can lead to patients shouldering the higher costs associated with not only needing to obtain prescriptions from specified pharmacies which may be more costly and less convenient, but also be subject to specific therapies being carved out of pharmacy coverage if the therapy is deemed too expensive by the PBM leaving the patient to shoulder the cost. For all of these reasons, ERISA should better consider the definition and coverage of specialty drugs under its purview.

Despite the costs associated with specialty drug coverage, however, we do believe there are a number of ways in which specialty drug costs could be better managed on behalf of plans and the patients they serve.

Reinsurance models can be used to reduce barriers to amortizing value-based payments, particularly across plan years and even portably across plans. All payers, regardless of whether they are public, commercial, or self-insured, are challenged by paying millions of dollars up front for a high cost specialty drug without potential cost-recovery if the therapy is less effective than desired and/or the patient departs the plan and the future savings derived from better health are realized by a different payer altogether. We believe commercial payers, and particularly self-insured plans, would benefit greatly from a robust approach for amortizing value-based payments across plan years and across plans, and the federal government in supporting these various models could be instrumental in creating such an approach.

Another potential tool employers can use to expand the risk pool and lower costs would be through the adoption of a value-based purchasing agreement. The best price under a value-based arrangement is the maximum possible price paid, assuming all patient outcome benchmarks are satisfied. If said benchmark is not satisfied, then the purchaser recoups some of the cost paid for the therapy. This does not prevent an insurer from pursuing rebates or other price concessions under a value-based arrangement if the treatment fails to meet its benchmarks, it simply allows for the risk of the cost of the therapy to be spread across parties. We are beginning to see positive data returned in the use of this model through therapies for sickle cell disease,[8] and it could represent another cost-saving measure for both insurers and beneficiaries.

In closing, if the fiduciary responsibilities under ERISA are updated to reflect the new realities of the integrated healthcare system we now live in and other reinsurance and risk pooling models are considered in insurance markets, we could see more robust and affordable coverage for all patients. We greatly appreciate the opportunity to provide feedback on the Chairwoman's consideration of how ERISA may be improved to increase access to quality insurance for the NMD community. Should you need any further information please contact Joel Cartner, Director, Access Policy at jcartner@mdausa.org.

[8] Allen, Jeremy et al. "Medicaid coverage practices for approved gene and cell therapies: Existing barriers and proposed policy solutions." *Molecular therapy. Methods & clinical development* vol. 29 513-521. 16 May. 2023, doi:10.1016/j.omtm.2023.05.015

Sincerely,

Joel Cartner, Esq
Director, Access Policy
Muscular Dystrophy Association