Statement from Muscular Dystrophy Association:

MDA Calls for Widespread Insurance Coverage of FDA-Approved Neuromuscular Disease Treatments

March 8, 2023

Today, the Muscular Dystrophy Association (MDA) is joining with the neuromuscular disease community to speak out for better access to FDA-approved therapies for patients. Unfortunately, insurers continue to deny coverage of FDA-approved therapies for those with neuromuscular diseases at an unreasonable and harmful rate. With so few FDA-approved therapies available for those living with neuromuscular diseases, every insurance denial means more time must be spent finding new ways to access care. Many neuromuscular diseases are progressive, and while an approved therapy may slow or stop a patient's progression, no therapy can reverse what a patient has lost while waiting for appropriate care.

Most recently, MDA has taken notice of Cigna's decision to deny coverage of the ALS drug Relyvrio. However, MDA has also noted denials of care for, among others, Spinraza and Zolgenzma for spinal muscular atrophy as well as Emflaza and exon skipping therapies for Duchenne muscular dystrophy. Anytime access to an FDA-approved treatment for our community is denied, MDA stands staunchly opposed to such decisions and urges each insurance provider to reconsider their decision.

Denials of coverage occur across all types of insurance. As an example of the negative effects of these coverage decisions, consider a baby born with Spinal Muscular Atrophy (SMA) in Texas a few years ago.[1] She was prescribed Zolgensma; however, her claim under one of Texas' Medicaid providers was denied. This decision left her family with a choice, accept the denial and a bill of around 2 million dollars or not receive the therapy at all. This delay in treatment left their daughter facing increasing irreversible declines in her health as she waited for an effective treatment. While the decision, in this case, was eventually reversed, unfortunately, situations such as this one are becoming more common.

In addition to coverage denials under Medicaid or Medicare, coverage denials are also increasingly prevalent in Affordable Care Act (ACA) Marketplace plans and employer-sponsored plans. Pharmacy Benefit Managers (PBMs) often determine what therapies are covered based on proprietary pricing determinations or formularies of covered medications on

[1] SMA Story

behalf of health insurers, negotiate rebates and discounts from drug manufacturers, and contract directly with individual pharmacies to reimburse for drugs dispensed to beneficiaries. The negotiating power PBMs wield combined with the opacity of the process can actually disincentivize manufacturers from lowering prices. These factors result in higher prices for patients or denials of coverage based on those prices.

Employer-sponsored health plans have begun more aggressively eliminating coverage for specific disease areas and drug classifications, particularly for those living with Duchenne muscular dystrophy. Insurers implement these denials of care by instituting arbitrary standards for coverage, such as requiring a patient to be ambulatory for authorization of treatment. Another way in which health insurers diminish access to care is through their use of Quality Adjusted Life Years (QALYs). QALYs are used in health economic evaluations to quantify the health effect of a medical treatment and help payers allocate resources. However, the use of QALYs to determine the benefit of treatments for patients is flawed, as the "quality of life" metric relies on an inherently ableist and utilitarian concepts and assumes outcomes for able-bodied patients in perfect health.

While many of these developments are concerning, MDA is taking action. With our partners in the Partnership to Protect Coverage we are tackling health insurance coverage adequacy by advocating for greater access to standardized health plans and Essential Health Benefits,[2] nondiscrimination in health plans under the ACA[3], and health equity[4] under Medicare and Medicaid. These actions will help ensure our community obtains quality insurance and affordable FDA-approved therapies. We also recently urged the Federal government to limit the use of prior authorization to only when it is absolutely necessary. Limiting prior authorization ensures timely access to care and FDA-approved therapies.[5] MDA is leading the charge on efforts to ban the use of discriminatory QALYs[6], and is looking for further opportunities to tackle problematic PBM and employer-sponsored insurance practices.

MDA is actively engaged in discussions with all stakeholders, including insurers and biopharmaceutical manufacturers, to ensure affordable access to FDA-approved therapies for all neuromuscular disease patients. MDA is even funding our own research into the subject of denials of care across the disability community to ensure effective access to care.[7] We will continue to work with partnering organizations in the Partnership to Protect Coverage and Medicare Access for Patients Rx to ensure patients' access to care is protected. MDA will be relentless in our advocacy to ensure access to FDA-approved therapies for the neuromuscular disease community.

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[2] Response to Request for Information re: EHB
[3] Statement on Section 1557 (nondiscrimination in healthcare)
[4] Statement on Health Equity
[5] Response to Request for Information re: EHB
[6] Letter to Congress on QALY-ban Legislation
[7] MDA Announces Advocacy Collaboration Grants